

Characterizing Patient Engagement in Research Funded by
the Patient-Centered Outcomes Research Institute and
Exploring the Moral Importance of Patient Engagement in
Research

by
Lauren Elizabeth Ellis, M.A.

A dissertation submitted to Johns Hopkins University in conformity with the
requirements for the degree of Doctor of Philosophy

Baltimore, Maryland
June, 2015

© 2015 Lauren Elizabeth Ellis
All Rights Reserved

Abstract

Patient engagement in research includes a range of activities in which researchers involve patients in ways other than as subjects of research. Research funders and researchers are increasingly recognizing patient engagement as a strategy that can lead to beneficial effects including the production of research that is more relevant to patients or of higher quality. The Patient-Centered Outcomes Research Institute (PCORI), a large research funding organization Congressionally authorized under the Patient Protection and Affordable Care Act, requires engagement of patients and other stakeholders in its funded comparative effectiveness research. Despite growing support for engagement, there is little empirical research and little conceptual scholarship examining patient engagement.

This dissertation seeks to advance current understanding of patient engagement through three aims. Aim one seeks to characterize researchers' experiences with patient engagement in research funded by PCORI, and aim two seeks to characterize patients' experiences being engaged in PCORI-funded research. To address these empirical aims, interviews were conducted with both researchers and with the patients they were engaging in their PCORI-funded research.

The results of these aims are reported in two papers. The first describes patient engagement in PCORI-funded projects including how, when, and why patients were engaged, and the extent to which patient input was reported to impact the relevance, feasibility, acceptability, and quality of the research. Findings suggest that the particular approach researchers use to engage patients may be less relevant to achieving desired outcomes than the manner in which engagement strategies are implemented.

The second empirical paper focuses on challenges to and successful strategies for patient engagement as reported in interviews. This paper also provides suggested actions to address challenges and bolster infrastructure for engagement including modifications to institutional policies, development of programs and researcher networks, and provision of resources and training.

Aim three explores the value of patient engagement from a normative perspective and ascertains the circumstances in which patient engagement is morally important. Drawing on interview findings, this conceptual paper analyzes the moral importance of the instrumental effects of engagement—namely, enhanced relevance, accountability, and respect—and whether engagement can be said to have intrinsic moral value.

Primary Readers: Nancy Kass, ScD (Advisor)

Kay Dickersin, PhD, MA (Chair)

Holly Taylor, PhD, MPH

Maria Merritt, PhD

Alternate Readers: Brendan Saloner, PhD

Katherine Clegg Smith, PhD, MA

Acknowledgments

I would not have been able to accomplish this project without a number of people who provided guidance and support throughout the process and for whom I am incredibly grateful. Chief among them is Dr. Nancy Kass. Nancy provided continuous feedback and support and challenged me as a scholar in bioethics and as a researcher. She also encouraged me to explore and pursue many opportunities while in the PhD Program. I greatly admire Nancy not just as an intellectual but also as a kind and considerate person, and I am honored to have such a wonderful mentor.

I am also very grateful for the members of my dissertation committee and thesis readers who each provided me with a wealth of advice and encouragement and made themselves available to discuss my work and review drafts of my manuscripts. I am thankful for the several discussions I had with Dr. Maria Merritt, which helped me to develop and refine my conceptual work, and for her careful review of my conceptual paper draft. I am also grateful for Dr. Holly Taylor, who reviewed my empirical papers with short notice and provided valuable guidance on my approach. Dr. Kate Smith gave me advice on my qualitative analysis and challenged me to sharpen my thinking on themes that emerged from my data. Last, I appreciate the thoughtful feedback that Dr. Kay Dickersin gave me on my work and her continued support of my research interests in patient engagement.

Thanks is also owed to Dr. Jodi Segal and Dr. Albert Wu who served on my dissertation proposal committee and provided comments that helped shape the design and execution of this project. I also want to thank Dr. Stephanie Moraine and Dr. Travis

Rieder for graciously taking the time to discuss my work on multiple occasions, review my conceptual paper, and provide helpful comments.

I would like to acknowledge the important role that members of the Johns Hopkins Community Research Advisory Council (C-RAC) have had in this work. Not only did the C-RAC review my project and provide feedback on my interview guides and questionnaires, they also welcomed me to serve as a member of the research review committee, which has been a rewarding experience for me. My cognitive interview participants also helped me improve my interview guides. All the investigators and patients who were willing to share their experiences as part of the interviews I conducted for this research also deserve recognition. I truly enjoyed meeting and interviewing study participants and learning from them.

I would also like to thank the Patient-Centered Outcomes Research Institute (PCORI). During the time I worked on this project, I was also a part-time employee of PCORI. I am grateful to PCORI for their encouragement for this project's conduct and completion. In particular, I would like to thank Dr. Lori Frank and Dr. Laura Forsythe for providing me the opportunity to further develop my content expertise in patient engagement through my experience working with them.

Last, I would like to thank my family and friends. I will always be grateful for the numerous ways in which my parents have supported me and helped to cultivate my intellectual interests. I am so thankful for my husband, Alden Ellis, who has been and remains my champion. At times when I was discouraged, he was always there to celebrate my progress, encourage me, and make me a delicious dinner. I am so glad for the wonderful friends I have made here at Hopkins including Carleigh Krubiner, Molly

Simmons, Danielle Whicher, Leila Jamal, and Susan Yeh, among many others. You have all enriched the last five years of my life, both socially and intellectually, and I am thankful to have you all as my colleagues and friends.

Table of Contents

MANUSCRIPT 1: Characterizing Patient Engagement in PCORI-Funded Research to Enhance Understanding of Its Ethical and Practical Value: A Qualitative Study of Principal Investigators and Patients	1
ABSTRACT	1
INTRODUCTION	2
METHODS	4
RESULTS.....	7
DISCUSSION	17
CONCLUSION.....	21
TABLES AND FIGURES	23
REFERENCES.....	30
 MANUSCRIPT 2: Challenges to and Successful Strategies for Patient Engagement in Research.....	33
ABSTRACT	33
INTRODUCTION	34
METHODS	35
RESULTS.....	38
DISCUSSION	48
CONCLUSION.....	52
TABLES	53
REFERENCES.....	55
 MANUSCRIPT 3: When and Why is Patient Engagement in Research Morally Important?	58

ABSTRACT	58
INTRODUCTION	59
METHODOLOGICAL APPROACH	62
INSTRUMENTAL VALUE OF PATIENT ENGAGEMENT	64
NON-INSTRUMENTAL VALUE OF PATIENT ENGAGEMENT	86
CONCLUSION.....	91
TABLES	95
REFERENCES.....	96
APPENDICES.....	101
APPENDIX 1: Johns Hopkins Bloomberg School of Public Health IRB Notices.....	101
<i>A: Original IRB Approval Notice.....</i>	<i>101</i>
<i>B: IRB Amendment Approval Notice</i>	<i>103</i>
APPENDIX 2: Extended Methods for Empirical Study	106
APPENDIX 3: Initial Recruitment Emails.....	126
<i>A: Initial Recruitment Email for Principal Investigators</i>	<i>126</i>
<i>B: Initial Recruitment Email for Patients.....</i>	<i>128</i>
APPENDIX 4: Participant Consent Forms.....	130
<i>A: Consent Form for Principal Investigators</i>	<i>130</i>
<i>B: Consent Form for Patients</i>	<i>133</i>
APPENDIX 5: In-Depth Interview Guides	135
<i>A: In-Depth Interview Guide for Principal Investigators</i>	<i>135</i>
<i>B: In-Depth Interview Guide for Patients</i>	<i>138</i>
APPENDIX 6: Participant Questionnaires.....	140
<i>A: Questionnaire for Principal Investigators.....</i>	<i>140</i>
<i>B: Questionnaire for Patients</i>	<i>142</i>

APPENDIX 7: Coding Scheme for Qualitative Analysis	145
APPENDIX 8: Community Research Advisory Council (C-RAC) Review.....	153
<i>A: Feedback from C-RAC Research Review Committee</i>	<i>153</i>
<i>B: Response to Feedback from C-RAC Review</i>	<i>157</i>
APPENDIX 9: Lay Person Summary	160
CURRICULUM VITAE	164

List of Tables

1.1: Background Characteristics of PIs

1.2: Background Characteristics of Patients

1.3: Characteristics of Engagement in Projects in Study Sample

1.4: Patient and Investigator Quotations on the Effects of Engagement at Various Points
During the Research Process

2.1 Overview of Recruitment and Enrollment Numbers

2.2 Characteristics of Study Participants

2.3 Select Quotations from Interviews

3.1 Overview of the Moral Reasons for PE (Patient Engagement) in Research

A.1: Code Names and Definitions

A.2: C-RAC Feedback on Patient Questionnaire

A.3: C-RAC Feedback on Patient Interview Guide

A.4: Executive Summary of Discussion During C-RAC Research Review

A.5: Interview Questions That Have Been Deleted

A.6: Interview Questions That Have Been Rephrased

A.7: Jargon Terms That Have Been Removed from the Interview Guide

List of Figures

1.1: Recruitment Flowchart for Pilot Projects

1.2: Recruitment Flowchart for Cycle I Awards

MANUSCRIPT 1: Characterizing Patient Engagement in PCORI-Funded Research to Enhance Understanding of Its Ethical and Practical Value: A Qualitative Study of Principal Investigators and Patients

ABSTRACT

Background: There is growing interest in patient engagement in research from both ethical and practical standpoints, yet there are few empirical investigations of the nature of patient engagement and its effects. This information is needed to advance discussions on the ethical and practical value of patient engagement and when it ought to be conducted. *Methods:* The aim of this study was to characterize patient engagement in research funded by the Patient-Centered Outcomes Research Institute (PCORI) as a step toward enhancing current understanding of how patient engagement is operationalized and its effects. Qualitative interviews were conducted with 19 PCORI-funded principal investigators and with 33 patients engaged in 18 of the same 19 projects. *Results:* Reasons cited for engaging patients included to enhance relevance and feasibility and to improve dissemination. While engagement occurred at different points during the research, patients were most commonly engaged in reviewing study materials and less commonly engaged at earlier points. Engagement varied by approach, frequency of interaction, and the extent to which patient input changed the research and was reported to impact the relevance, feasibility, acceptability, and quality of the research. *Conclusion:* These findings suggest that researchers and funders should focus less on particular engagement approaches and more on how patients can best influence decision-making, as this tracks with achievement of impacts of engagement on the research process. While more research is needed to evaluate the effects of engagement, these findings offer insights into the kinds of influence that engagement can have on the research process and thus the ethical and practical arguments that may motivate researchers to engage patients.

INTRODUCTION

Patient engagement in research refers to researchers involving patients in research in ways other than as subjects of research (Hanley et al., 2003) and includes activities falling anywhere on a spectrum from low levels of engagement such as single consultations with patients to patient-researcher collaborations or even patient-led research (Staley, 2009).

There is growing interest in patient engagement. The Institute of Medicine (2009) identified engagement of patients and stakeholders as a priority for comparative effectiveness research (CER). This priority is exemplified in the Patient-Centered Outcomes Research Institute (PCORI), a private, nonprofit research organization that was Congressionally authorized under the Patient Protection and Affordable Care Act (2010) to fund CER that engages patients and other stakeholders (PCORI, 2014). With over \$730 million awarded to a total of 365 research projects to date (PCORI, 2015) and plans to fund over \$3 billion in research on a wide range of topics by the end of this decade (Washington & Lipstein, 2011), PCORI's research portfolio has resulted in an unprecedented commitment to patient engagement in the United States.

Increased support for patient engagement is fueled by the belief that it may have practical benefits on the research process including enhanced relevance of research to patients, higher quality research, and improved uptake of research findings (Caron-Flinterman, Broerse, & Bunders, 2005; Roehr, 2010; Selby, Beal, & Frank, 2012; Sox & Greenfield, 2009). These practical benefits are also significant from a normative perspective. For example, higher quality research may be more useful to patients, more socially valuable, and a better use of limited resources. Further, patient engagement may

be ethically important for other reasons such as a mechanism to hold researchers and funders accountable or as a way to show respect to patients (Boote, Telford, & Cooper, 2002; Gradinger et al., 2013; Tritter & McCallum, 2006). To the extent to which any of these practical and ethical reasons for engaging patients in research are compelling, it becomes important to understand how patients are being engaged in research and the effect of their engagement on the research process.

A number of recent literature reviews provide emerging evidence in support of some of the hypothesized effects of engagement (Brett et al., 2014; Concannon et al., 2014; Domecq et al., 2014; Forsythe et al., 2014; Shippee et al., 2013); however, these literature reviews are limited by several factors including the absence of indexing terms for literature searches on engagement, the lack of uniform reporting on engagement, and insufficient details reported or underreporting about engagement approaches and their effects. Further, there have been no empirical investigations specifically on PCORI-funded studies, which are bringing a new magnitude to engagement. Empirical studies examining the nature of engagement approaches, the effects of engagement, and which approaches appear most impactful remains an area of need (Brett et al., 2014; Concannon et al., 2014; Snape et al., 2014; Staniszewska et al., 2011; Workman, Maurer, & Carman, 2013). This qualitative study was undertaken as a step toward enhancing current understanding of patient engagement, including the practice of patient engagement and the nature and degree of the effects of engagement, all of which are relevant to arguments on the ethical and practical value of patient engagement in research. The research question of this qualitative study was: “how was patient engagement operationalized in the first set of PCORI-funded projects?”

METHODS

The objective of this study was to characterize experiences with patient engagement in PCORI-funded research. The approach to data collection and analysis followed a qualitative descriptive approach which aims to generate a comprehensive description of the event under investigation (Sandelowski, 2000). This study was approved by the Johns Hopkins Bloomberg School of Public Health Institutional Review Board (JHSPH IRB).

Sampling

Purposeful sampling from PCORI's first two rounds of funding awards—the Pilot Projects Grant Program (50 two-year projects awarded in June 2012) (PCORI, 2012a) and the Cycle I Awards (25 three-year projects awarded in December 2012) (PCORI, 2012b)—was conducted as these projects were closest to completion at the time this study was initiated. Funding applications were reviewed to aid the sampling process. Only studies with funding applications describing plans for patient engagement—defined as the involvement of patients, patient representatives, community members, and/or caregivers of patients in a way other than as subjects of research—were eligible for inclusion.

The goal of purposeful sampling was to include typical engagement approaches described in the funding applications while also capturing variation with respect to other project attributes relevant to engagement that were also described in the funding applications, including: the population engaged (e.g., healthy patients, sick patients, patients from advocacy organizations, lay patients, minority patients, elderly patients), whether researchers had a previous relationship with the patients they were engaging,

whether projects were also engaging non-patient stakeholders (e.g., clinicians, policymakers, purchasers, payers), and the study topic (e.g., behavioral health, chronic disease, rare disease). Eligible projects were grouped by the kinds of engagement approaches described in the applications, including: advisory groups, focus groups/interviews, surveys, patient co-investigators, patient research team members, pilot testing, and novel methods. The sample was built to ensure each approach was included and that there was variation with respect to the other characteristics described above.

When principal investigators (PIs) were recruited, they were asked their willingness to (i) participate in an interview themselves and (ii) refer up to two patients who were actively engaged in their projects and had somewhat different backgrounds or experiences (e.g., affiliation with advocacy organizations, health status, experience with study topic, experience with engaged research, age, gender) for possible interviews. PIs were asked to first seek patients' permission to share contact information with investigators of this study, and then, if patients were willing, contact information was forwarded to investigators.

Interviews and Questionnaires

Oral consent was obtained before each interview. During both the recruitment and informed consent processes, interview participants were informed that the interviewer (LE) worked at PCORI and that PCORI was aware of but had no involvement in the project. Participants were also told that the project comprised the research for the student's doctoral dissertation, and that PCORI would have no access to any information about which studies or individuals were recruited or interviewed, nor would PCORI have access to any data collected as part of the research.

All interviews were audio-recorded. Interviews adhered to a guide covering four domains: background information about the project and nature of engagement activities, the purpose and goals of patient engagement, experiences being involved in the engagement activities, and general beliefs about patient engagement. However, respondents were free to discuss topics within these domains that were of greatest relevance to them. Participants also completed a short questionnaire with demographic and background questions.

To assess the appropriateness and understandability of interview questions, two cognitive interviews following the approach outlined by Beatty and Willis (2007) were conducted. One was with a Johns Hopkins Hospital researcher with experience engaging patients and the other was with a patient involved in a community advisory board through Johns Hopkins Hospital. Interview and questionnaire items were also reviewed by a community research advisory council, and changes were made in response to community feedback.

Analysis

Data collection and analysis occurred in an iterative fashion, with interviews conducted until informational redundancy was reached. The thematic analysis relied on a combined deductive and inductive approach. Audio-recordings were transcribed and reviewed for accuracy. Questions from the interview guide informed development of an initial coding scheme, with inductive codes added as transcripts were reviewed. Codes were organized into thematic families, applied to a subset of transcripts, and further refined before a finalized coding scheme was applied to all transcripts.

A second coder was trained and coded six transcripts. The double-coded transcripts were reviewed and percent agreement of the codes was calculated to assess the reliability of the scheme. A high level of concordance (80% agreement) was achieved. Questionnaire data were analyzed to consider participant demographics, and the software program ATLAS.ti (1999) was used during analysis.

RESULTS

This section reports study findings. The findings from the questionnaire include background and demographic information about study participants. The findings from the interviews consist of details about patient engagement in the projects including the reasons why researchers engaged patients in projects and reasons why patients decided to become engaged in the projects, the types of engagement approaches used in the projects, how relationships between researchers and patients were established, when in the research process engagement took place, and the key contributions of engagement. Throughout this section, interview quotations are labeled by funding cycle (“PP” for Pilot Project; “C” for Cycle I), study number (PP1-PP9; C1-C10), and respondent type (“PI” for investigator; “Pt A” or “Pt B” for the first or second patient interviewed from a given study).

Participants

Qualitative interviews were conducted with 19 PIs from 19 different PCORI projects and 33 patients from 18 of the same 19 projects. Interviews were conducted between December 2013 and May 2014.

Pilot Projects that engaged patients (42 of 50) were eligible for this study.¹

Nineteen Pilot Project PIs were contacted to see if they were willing to participate and to refer patients for interviews (Figure 1.1). Nine of the 19 Pilot Project PIs enrolled in the study, and 10 declined participation: one did not respond after four attempts and the others cited reasons including lack of time (n=3), IRB concerns (n=1), not yet engaging patients (n=1), not willing to have patients interviewed (n=2), and no longer able to contact patients whom they had already engaged (n=2). All 25 Cycle I Awards engaged patients and thus were eligible. Fourteen PIs from Cycle I Awards were recruited (Figure 1.2). Ten of the 14 enrolled in the study, and four declined participation: one did not respond after four attempts and the others declined because of lack of time (n=1) and not willing to have patients interviewed (n=2). A total of 19 interviews were conducted with PIs, 11 in-person and 8 by phone. The mean length of PI interviews was 45 minutes (range: 27 to 67 minutes). Eighteen Pilot Project patients were recruited; 16 participated and two declined because of lack of interest. Nineteen Cycle I Award patients were recruited; 17 participated and two declined: one cited lack of time and there was no response from the other. A total of 33 patients were interviewed, 14 in person and 19 by phone. The mean length of patient interviews was 42 minutes (range: 16 to 62 minutes).

Of PIs interviewed, half were female; the majority were white, and ages ranged from under 30 to over 61 years (Table 1.1). Nearly all Pilot Project PIs had engaged patients in prior studies, but few Cycle I PIs had. Most PIs had been on faculty for at least six years and had served as PI on at least four grants, including their PCORI project.

¹ As early, methods-focused studies, the Pilot Projects did not all engage patients and were also not required to be comparative, unlike the Cycle I Awards which were comparative studies.

Over two-thirds of patients interviewed were female; the majority were white, with a wide age range represented (Table 1.2). Over two-thirds were employed and held a college or graduate degree, and about half reported having a chronic condition. While over a third had previously engaged in research, more patients from the Pilot Projects had previous experience compared to Cycle I Awards.

Reasons for Patient Engagement

PIs and patients both reported getting patient input as a general goal of patient engagement; however, PIs tended to further specify that such input was valuable for multiple specific reasons. The main reasons reported by PIs were to enhance the relevance of research, enhance feasibility, improve dissemination, and fulfill PCORI's requirement. Patients also described increased relevance as a main reason for patient engagement.

Relevance: About two-thirds of investigators (n=13) and nearly half of patients (n=15) described engaging patients to enhance relevance, making it the most commonly cited reason. PIs viewed engagement as a way to understand patients' needs so that research could address them: "I think the main reason is just to find out what matters to them" (PI, C10). Patients expressed the same idea: "If you leave the user out of the process, then you deliver a product that isn't going to meet their needs as well (Pt A, PP7).

Feasibility: Just over half of investigators (n=10) described engaging patients to enhance feasibility. This included engaging patients to identify the most practical research processes and to anticipate challenges. Investigators often described relevance

and feasibility together: “It has helped us to design a process, both process and content, that is much, much more usable and meaningful to patients, which is the goal” (PI, C2).

Dissemination: About half of investigators (n=9) described engaging patients to improve dissemination, such as to ensure results were understandable and would reach the intended audience: “One of the goals of the [advisory group] is to be able to understand the results, interpret the results, and then be able to explain the results to other people” (PI, C3).

Requirement: Lastly, about half of investigators (n=9) cited PCORI’s requirement as a reason, though not necessarily the main reason, they engaged patients in their PCORI project.

Why Patients Became Engaged

Patients articulated a diverse set of motivations for becoming engaged in research including to reciprocate benefits they had received from the investigator’s institution, to improve the patient community’s health, to improve one’s own health, and to learn more. Patients often discussed multiple reasons for joining projects: “Not only did I think that maybe I had something I could contribute, but that also I would be learning as well” (Pt A, PP9). Patients with preexisting relationships with PIs cited that as a reason for becoming engaged: “I did it because [researcher] asked me to do it and that was it... It was a personal relationship” (Pt A, PP8).

Types of Engagement

A range of approaches to engagement was included in the sample, and PIs and patients described these various approaches: advisory groups, patient co-investigators,

patient research team members, pilot testing, focus groups, interviews, surveys, and novel methods (Table 1.3). About three-quarters of projects (n=14) used more than one approach.

Advisory Groups: Used by almost all of the projects (n=16), advisory groups were the most commonly used approach that informants in the sample described. This included community advisory boards (CABs), patient and family advisory councils, and multi-stakeholder steering committees. PIs and patients described the frequency with which these groups met for their projects; meetings ranged from monthly to annual, with quarterly meetings the most common. Advisory group meetings typically included activities such as researchers providing progress updates, presentations and discussions on study-related topics, and requests for feedback on specific areas of the research. Investigators described advisory groups as people “who we can go to and say, ‘Hey, what do you think about this approach? What do you think about this method? How should we say this?’” (PI, PP1).

Patient Research Team Members: About half of projects (n=9) involved patients as members of the research team, including as co-investigators, making it the second most common approach described by interview participants. Patient research team members reported that they met regularly with scientific investigators, provided input throughout the research, and often connected PIs to other patients or led advisory group meetings. A typical PI statement about a patient in this role describes the patient as “critical, not only to provide the patient perspective when we were developing and writing the proposal, but also to connect us with other stakeholder groups” (PI, C5). Similarly, a patient co-investigator described her leadership role: “[It] was stated in the

grant proposal that I would be leading the patients/family advisory board, and so one of my tasks is to be the chair of that board” (Pt A, PP3).

Pilot Testing: Patients and researchers from about half of projects (n=9) describing engaging patients in pilot or usability testing of study interventions or materials to see how user-friendly, feasible, or understandable they were. As a patient explained: “Once the iPad application was ready to be used with the patients, we were given it for a couple of weeks to play with and practice, and then go through all the uses that a patient would use it for. So we gave some feedback on that” (Pt B, PP9).

Focus Groups, Interviews, and Surveys: While less common than the use of other methods, focus groups/interviews were used by about a third of projects (n=7), and surveys were used by just a few projects (n=3). These methods to engaging patients usually involved single interactions to get input in targeted ways such as discussing domains for inclusion in study instruments, ranking outcomes of greatest priority, or designing the study intervention.

Novel Methods: A few projects (n=3) used novel methods to engage patients. These methods included an interactive web-based discussion forum to get patient input on study materials, a multi-stakeholder engagement workshop to help design the study intervention, and a PI-created in-person presentation and discussion forum to get patient input throughout the research process.

How Relationships were Established

PIs and patients from over half of the projects (n=11), mostly Pilot Projects, described having preexisting relationships, often from prior engagement or professional

interactions including working at the same institution or shared membership in an organization. Investigators sought new relationships with patients through referrals from healthcare providers, clinics, national patient associations, local disease group leaders, clinician co-investigators, or other patients. About a quarter of PIs (n=5) reported recruiting participants from their PCORI project to serve as some of their engagement partners, while about a third of PIs (n=6) described recruiting engagement partners from existing groups, such as the hospital's patient advisory council.

Engagement by Stage of Research

Patients were engaged at different points during the research. The majority of both PIs (n=16) and patients (n=22) referred to patients being involved in reviewing or developing study instruments, consent documents, recruitment materials, or intervention-related materials. Patients and PIs also commonly mentioned engagement in study design and methods.

Beyond this, patients and researchers from each project described patients being engaged in usually just one or two of the following several areas: proposal development, study interventions, study outcomes, recruitment, and data analysis/interpretation. For each area, about half of studies reported engagement. Projects varied regarding in which of these areas they engaged patients.

Engagement was uncommon at the earliest stages of research. Only a few researchers (n=3), all from Pilot Projects, described engaging patients in research question development. No patients commented specifically on being engaged in research question development, although patients from one study described participating in early meetings to “frame the whole issue” (Pt A, PP5).

Both researchers and patients provided many specific examples of engaging patients throughout the research process and the effects of such engagement. Quotations from researchers and patients illustrating various effects of patient engagement at different points in the research process are depicted in Table 1.4.

Key Contributions of Engagement

Patients and PIs described their views on how patient engagement contributed to the research. What PIs perceived as the main contributions of engagement matched some of the reasons they cited for engaging patients including to enhance relevance and to improve feasibility. Researchers and patients also described improved appropriateness and acceptability of the research and increased research quality as effects of patient engagement. Patient responses were generally not as detailed as PI responses, but their responses were consistent with the substance mentioned by researchers.

Enhanced relevance

Enhanced relevance was the most common reason for engaging patients and was also described most frequently as the main outcome of engagement. Nearly all researchers (n=16) and about half of patients (n=16) described ways in which patient input impacted the relevance of the research including the relevance of study materials, interventions, and outcomes. About a third of PIs (n= 7) and a fourth of patients (n=9) noted that patient input enhanced the relevance of interventions. PIs described modifying interventions to better address patients' interests based on what they learned through engagement. One investigator reported that engaging patients and providers in designing the intervention "kept the patient needs front and center" (PI, C6). In an unusual

example, early discussions with patients led a Cycle I investigator to realize that patients were using a resource offered through local community centers in lieu of clinical treatment options, so the investigator added this community-based intervention as a third study arm.

Nearly a fifth of patients (n=6) specifically described being asked to give opinions on the relevance of outcome measures, and a third of PIs (n=7) described how conversations with patients confirmed the relevance of outcomes or prompted the addition of secondary outcomes that patients felt were important. For example, one PI stated, “[We] clearly needed to have very active engagement with patients to find out what they thought were important outcomes” (PI, PP3). A patient from that study confirmed this: “I think the consumer voice adds a lot to this, and we saw that especially with our outcomes” (Pt A, PP3).

Enhanced feasibility

About a third of patients (n=12) reported that their feedback enhanced study feasibility. A patient engaged in pilot testing of a novel intervention, for example, expressed this common belief: “The people that actually need the treatment, I think, can provide some pretty significant feedback in what works, what doesn’t work” for the intervention (Pt B, PP7). About a third of investigators (n=7) echoed this sentiment, describing how patient input helped them anticipate and solve challenges to transportation, compensation, and other study logistics.

Similarly, just less than half of PIs (n=8) attributed successful enrollment to patient input on recruitment materials and strategies: “I think probably the enrollment process has gone more smoothly because we had folks involved... I think that’s really

related to having a lot of patient input in terms of how to design the form and present the study to potential participants” (PI, C2).

Improved appropriateness and acceptability

With almost all of PIs reporting engaging patients in reviewing study materials (n=16), examples of changes to study materials prompted by patient input were described by over half of PIs (n=11) and just less than half of patients (n=14). Patients enhanced the appropriateness of study materials by providing input on the length of instruments, their comfort level answering certain questions, and the understandability of the language. A PI described patient feedback as prompting him to consider appropriateness for all kinds of patients: “What about patients who are lower in terms of socioeconomic status and lower health literacy? How is this going to apply to people who have significant disabilities? You know? She kept reminding us” (PI, PP2). Patient feedback of this nature was reported to spur changes to the wording and content of materials.

About a third of PIs (n=7) also reported that patient feedback confirmed the acceptability of study methods or prompted changes. For example, patients’ concerns about asking participants to share personal information in focus groups prompted an investigator to switch to individual interviews. Another investigator shortened the follow-up period and made arrangements for post-trial access to the experimental intervention after patient advisors expressed wanting all study participants to have an opportunity to benefit.

Improved quality

Often contrasting their PCORI projects with earlier research, about a third of PIs (n=7), mostly from Pilot Projects, suggested that their projects were better because of engagement. As one investigator expressed:

You are going to come up with a better design for that survey if you're engaging patients in the process. And maybe because I'm old enough to have seen the old method of doing this, which is where the professors come up with the survey, and then watch people either not fill it out or give you invalid data because it's—from their perspectives—stupid questions or questions that are not really relevant or understandable to them. (PI, PP6)

A few PIs (n=3) linked other effects of engagement—namely, enhanced relevance and appropriateness of study materials—with subsequent effects on research quality, noting that results are less accurate when patients do not understand questions asked of them. A few patients (n=5) also made this link: “It’s going to make the information gained from the research project more valid and more effective” (Pt A, C3).

DISCUSSION

This study of PCORI projects describes researchers’ and patients’ experiences and opinions regarding patient engagement in research. The findings provide insights into two areas identified previously as lacking sufficient evidence: the nature of patient engagement and its effects (Brett et al., 2014; Concannon et al., 2014; Snape et al., 2014; Staniszewska et al., 2011; Workman et al., 2013). As such, this study informs discussions on the importance of patient engagement from both practical and ethical perspectives.

This study found that patient engagement occurred at different points, both within given projects and across different projects. Further, in this sample, patient engagement also varied by approach, frequency of interaction, and researchers' responses to engagement across the various projects. Patients were most commonly engaged in reviewing study materials, while engagement early in the research was uncommon. PIs most commonly engaged patients in advisory boards, followed by as research team members, and lastly in other ways including focus groups, surveys, and pilot testing, all of which are documented methods (Concannon et al., 2014; Domecq et al., 2014; Staley, 2009). Frequency of interactions was remarkably varied but most commonly included at least quarterly meetings. Researchers' responses to engagement—that is, how engagement fit within the context of their project—ranged from using patient opinions to inform their decision-making to having patients play a greater role in decisions.

Investigators and patients in this study described how engaging patients in reviewing study materials and methods improved their appropriateness and enhanced the feasibility and relevance of the research. This suggests that patient engagement can achieve both ethical and practical goals and is consistent with other studies reporting similar influence (Brett et al., 2014; Concannon et al., 2014). Perhaps one reason why patients were most commonly engaged in reviewing study materials is because it is easier for patients who are unfamiliar with research to provide feedback on this generally less technical aspect of research. Alternatively, it may be that the stages at which patients are engaged inform the nature of the effects of patient engagement on the research process.

Researchers and patients reported enhanced relevance of the research as both a main reason for engaging patients and as a key contribution. The relevance of the

research to patients likely factors into patients' views of the social value of research, which is widely accepted as an ethical requirement of research (Emanuel, Wendler, & Grady, 2000); thus, these findings suggest that patient engagement may be a morally important strategy to increase the social value of research, at least from the patients' perspective. Despite the fact that relevance was commonly cited as a reason for engagement, researchers mostly did not engage patients early in the research process, where key contributions to the relevance of the overall research project could be made (Brett et al., 2014). Engagement throughout all phases of research including early in the process is a principle of community-based participatory research (Israel et al., 2003). Researchers may not have realized that engaging patients early in the process could enhance relevance, or they may have lacked time, support, and experience to engage patients at earlier points, especially prior to receiving funding (e.g., in formulating the research question and developing the grant proposal). Investigators accustomed to writing grants on their own, for example, may find it difficult to involve patients in that process, especially without dedicated resources for doing so. Given that relevance is both ethically and practically important and that early engagement may be key for enhancing relevance, funders should consider providing support for early engagement, perhaps even during proposal writing. Efforts to align funding mechanisms with the current grant writing process may facilitate early engagement as a means to produce research that is more relevant to patients.

In this study, researchers' responses to engaging patients varied independent of engagement approach and frequency of interaction. Some PIs involved patients in ways where patients could provide reactions and advice, while others described situations

where patients were more equal decision makers. This is comparable to what some in the field have classified as “consultative” versus “collaborative” levels of engagement (Hanley et al., 2003). Consultation refers to researchers asking for patients’ opinions but not being committed to follow them. By contrast, collaboration refers to an ongoing partnership, which involves sharing some control of the project with patients.

Arguably the purpose of engaging patients is for patients to influence the research, whether through advancing practical goals, ethical goals, or a combination of the two. Investigators and patients in this study gave multiple reasons for engagement, including to enhance the relevance and feasibility of research, which suggest the expectation for patient engagement to have some effect. A key question, therefore, is whether differences in engagement approach are relevant to the nature of, or degree to which, such influence occurred.

In this study, researchers’ responses to engagement—that is, their receptivity to patient feedback and the extent to which patients were involved in a leadership or decision-making role—as well as the frequency of interaction between researchers and patients seemed most related to the achievement of key practical and ethical contributions. While there is a need for research into whether certain effects of engagement are associated with different approaches, these findings suggest that the particular approach to engagement may be less relevant to achieving desired outcomes than the manner in which any number of engagement strategies are implemented. This suggests that researchers and funders ought to focus less on *types* of engagement and more on other characteristics, such as whether there is opportunity for patients to influence decision-making at certain points in the research, as this tracks with

achievement of goals of engagement. The particular reason for engaging patients in a given study is predictive of the kinds of influence that patients can have on the research.

There were several limitations to this study. First, it included only PCORI projects, which differ not only in their requirement for engagement but also in the support they receive for engagement. While this may limit the transferability of findings, the results may be relevant to other funders who may similarly introduce engagement as a requirement. Because half the investigators were new to engagement, the findings likely differ from more experienced investigators; however, the majority of investigators do not engage patients routinely, and thus findings may be relevant more broadly. Second, participating investigators may have had different experiences compared to those who declined participation, which may bias the results. There were a higher proportion of female investigators and double the proportion of investigators with senior academic rankings among refusing investigators compared to participating investigators; however, they were similar with regard to geographic region, academic degrees, and institution type (e.g., university, foundation, or other organization). Third, because projects were in progress, this study was unable to explore the effects of engagement on dissemination, where research is needed.

CONCLUSION

This study provides a rich description of patient engagement in PCORI projects, enhancing current understanding of how patient engagement is operationalized and what researchers and patients perceive as its main effects. Further, this study characterizes how, when, and why patients can be engaged in research, contributing to a limited

evidence base. Because reasons for engaging patients in research may be motivated by the anticipated effects of such engagement, knowing how patients can influence the research process is key to advancing arguments on the ethical and practical importance of patient engagement in research. Patient engagement may enhance the relevance, feasibility, acceptability, and quality of the research, all of which are important from practical and ethical perspectives. While future research is needed to develop objective measures to evaluate these effects of patient engagement and explore other possible ethically important outcomes of engagement (e.g., respect, accountability), this research offers insights into the kinds of influence that patient engagement can have on the research process and thus the ethical arguments that may motivate researchers to engage patients in their research.

TABLES AND FIGURES

Table 1.1: Background Characteristics of PIs (n = 19)	
Age	
<i>Under 30 years</i>	1
<i>31-40 years</i>	4
<i>41-50 years</i>	5
<i>51-60 years</i>	6
<i>61+ years</i>	3
Race/Ethnicity	
<i>African American (non-Hispanic)</i>	1
<i>Asian/Pacific Islander (non-Hispanic)</i>	1
<i>White (non-Hispanic)</i>	17
<i>Hispanic</i>	0
Gender	
<i>Female</i>	10
<i>Male</i>	9
Prior Experience	
<i>Engaged patients in research prior to this</i>	10
<i>Participated as a human subject in research</i>	16
Grants on Which Served as PI (Inclusive)	
<i>1-3 grants</i>	5
<i>4-6 grants</i>	5
<i>7-9 grants</i>	3
<i>10+ grants</i>	6
Years Serving on Academic Faculty	
<i><1 year</i>	0
<i>1-5 years</i>	4
<i>6-10 years</i>	4
<i>11-20 years</i>	5
<i>20+ years</i>	6

The source of these data is a short questionnaire completed by participants. All data are self-reported.

Table 1.2: Background Characteristics of Patients (n= 33)	
Age	
<i>Under 30 years</i>	3
<i>31-40 years</i>	1
<i>41-50 years</i>	4
<i>51-60 years</i>	12
<i>61+ years</i>	13
Race/Ethnicity	
<i>African American (non-Hispanic)</i>	4
<i>Asian/Pacific Islander (non-Hispanic)</i>	1
<i>White (non-Hispanic)</i>	26
<i>Hispanic</i>	2
Gender	
<i>Female</i>	23
<i>Male</i>	10
Prior Experience	
<i>Engaged in research prior to this</i>	13
<i>Participated as a human subject in research</i>	19
Highest Educational Attainment	
<i>Some high school, no diploma</i>	0
<i>High school graduate</i>	2
<i>Trade/technical/vocational training</i>	1
<i>Some college, no degree</i>	5
<i>College degree</i>	11
<i>Graduate degree</i>	14
Employment Status	
<i>Employed full-time</i>	16
<i>Employed part-time</i>	8
<i>Not employed, seeking employment</i>	1
<i>Not employed, not seeking employment</i>	8
Health Status	
<i>Chronic disease/condition</i>	17

The source of these data is a short questionnaire completed by participants. All data are self-reported.

Table 1.3: Characteristics of Engagement in Projects in Study Sample (n =19)	
Type of Engagement	
<i>Advisory group</i>	16
<i>Patient research team member or co-investigator</i>	9
<i>Pilot Testing</i>	9
<i>Focus groups/interviews</i>	7
<i>Surveys</i>	3
<i>Novel Methods</i>	3
<i>Used more than one engagement approach</i>	14
Populations Engaged	
<i>Patients or community members</i>	18
<i>Patient representatives from organizations</i>	6
<i>Caregivers</i>	7
<i>Also engaged non-patient stakeholders</i>	12
Prior Relationship Between Researchers and Patients	
<i>Yes</i>	11
<i>No</i>	8
Geographic Region	
<i>Northeast</i>	5
<i>Southeast and South</i>	6
<i>Midwest and West</i>	8

These data were derived from PI interviews. Novel methods included unique activities developed by the researcher: an interactive web-based discussion forum, an in-person multi-stakeholder workshop, and an in-person presentation and discussion forum. Pilot testing included patients testing study instruments or interventions to provide feedback on user-friendliness, understandability, etc.

Table 1.4: Patient & Investigator Quotations on the Effects of Engagement at Various Points During the Research Process	
Quotations from Investigators	Quotations from Patients
Research Topics/Question Development	
Our goal was to utilize [measures] which were able to cover many of these other domains of health and see whether they were able to reflect these areas that patients had told us were important... There was the patient engagement in terms of formulation of the question. (PI, PP2)	There are times in which the people in the community are the experts. So they are the experts on how we address the needs of [patient population]. They shape that conversation with us, we framed it out of conversations with the community advisory board. (Pt B, PP5)
Proposal Development	
There was a period of time when we were writing the grant and crafting the idea where the idea morphed because of the input of the team... Because we had an opportunity to engage a number of but not all parents in the process of crafting of the application, they already had ownership over it. (PI, PP1)	I wasn't there when [PI] formulated the idea in his head, but I was there just beyond...[PI] would do the main writing of the grant proposal...And then we would all have the document to make comments, suggested corrections or additions, deletions, etcetera, etcetera. (Pt A, PP3)
Study Design/Methods	
Our stakeholders really sort of actually came up with that design, corroborated with us that "Yes, that is a good design"... It allowed us to get at data in different ways, and to actually test –well, it's not really testing, but asking the stakeholder up front, "Would you have something to say about this? Could you spend an hour or two talking about your situation?" (PI, PP9)	We are also going to be interviewing, doing online methods... Even that involves a patient and a family voice, so that involves, actually, my son... meeting with [researchers] and saying, "Well, this is how I would feel comfortable communicating online. This is how I wouldn't be." And then going to our families and saying, "How would you feel comfortable communicating online?" (Pt A, PP1)
Study Materials	
It was going to be like an interview... The way it ended up happening is we now have all the participants do a written, self-directed portion, where they work through things on their own, and then there's a debriefing interview in the second half of it. That was a pretty substantial reworking of it, that went really well, but that was based on stakeholder feedback. (PI, C5)	So I think what I brought to the study were making our questions that we ask to a third grade reading level and also incorporating an aspect of the study where the questions could be read to the person through the computer, that there would be an audio, instead of just assuming people read... that's one of the components that I felt like I was able to bring. (Pt A, C2)
Study Intervention	
We started with, very broadly, "This is what the intervention will feel like as a patient or a family member; how does that sound?" And then we've been getting	Basically we've been involved from the beginning of discussions about what the procedures are and then we've looked at proposed documents for all of the various

really into the details where every month we'll have a representative...talk about the intervention in detail and get their feedback on the intervention. (PI, C3)	steps in the process... timing of the communication beforehand, involvement of the pharmacist as well as the nurses and social workers in that process. (Pt B, C3)
Study Outcomes	
This choice of secondary outcome measure being a measure of physical performance... That was informed by these community discussions. I would not have included that originally. That was not my intent. But after speaking to so many people that told me they wanted to be more active, I realized, I need to measure that. (PI, C9)	I think the biggest way that we've impacted the research is by giving our perspective. What might be an outcome that's desirable in the medical community could be completely different to a patient. And so we've been able to say where we were coming from, and I think that's really helpful. (Pt B, PP3)
Recruitment	
They've helped us out with everything from website design to the best ways to approach folks in the hospital. (PI, C8)	We also shared with him some other community resources... There's a small, local publication... They weren't aware of that, so I sent them the publication and the contact information. (Pt B, C9)
Analysis/Interpretation	
In terms of where we are now, analyzing and interpreting the data, I think they bring a lot into just kind of giving us a different perspective of how we're thinking about that. So when things don't come out exactly how we thought, they are like, "Oh yeah, but that may be because of A, B, or C." (PI, PP5)	Once the preliminary interviews were done... we were called in to just hear the assessment of the interviews and give them feedback on their summaries of the patient interviews. (Pt B, PP9)
Dissemination	
Just based on the diversity of the people in our board, I think there's going to be a lot of potential opportunities on both a very local level and then a more statewide level to disseminate our results. (PI, C1)	Even things of how we're going to publish and where we're going to publish things. I think that that really can be very helpful to have somebody who... knows those places where patients will read information and know that they'll understand. (Pt A, C5)

This table depicts select quotations from interviews with investigators (on the left) and patients (on the right) about patient engagement at various points during the research process. With the exception of Study Intervention, the investigator and patient in each row are not from the same study.

Figure 1.1: Recruitment Flowchart for Pilot Projects

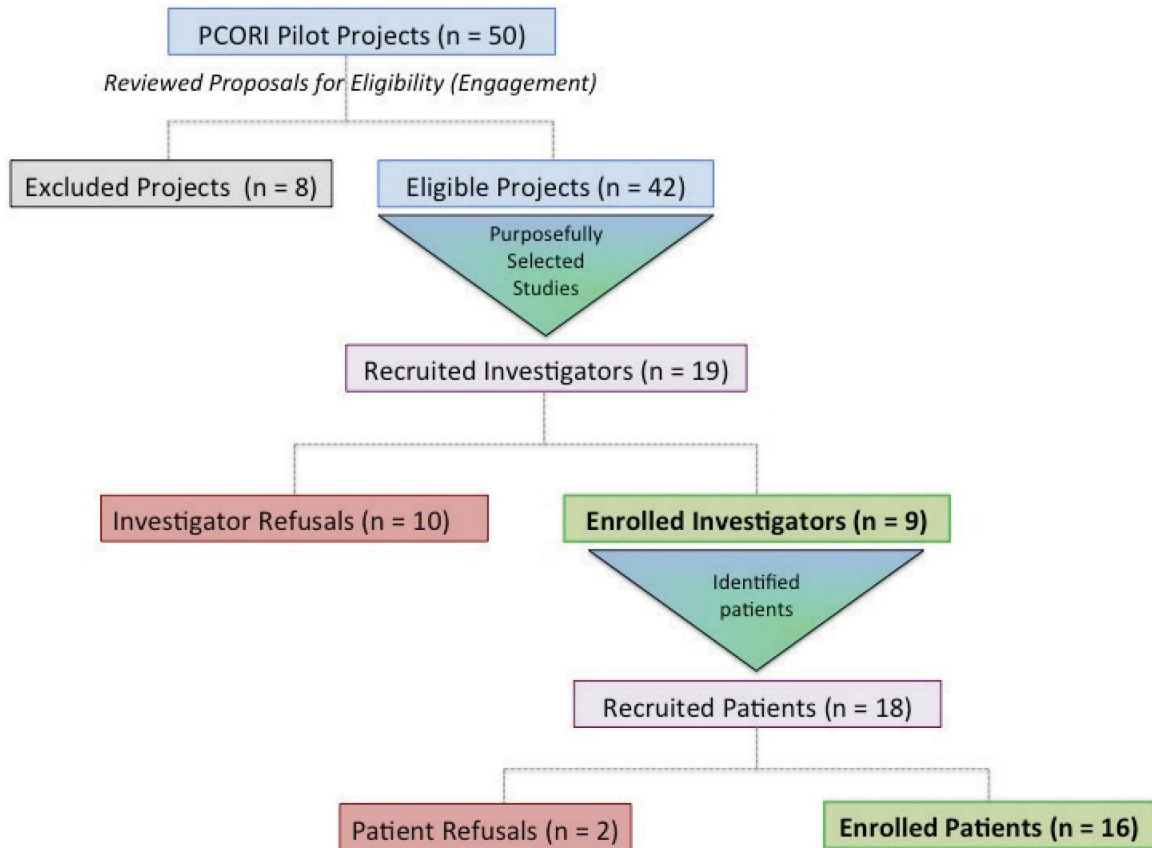
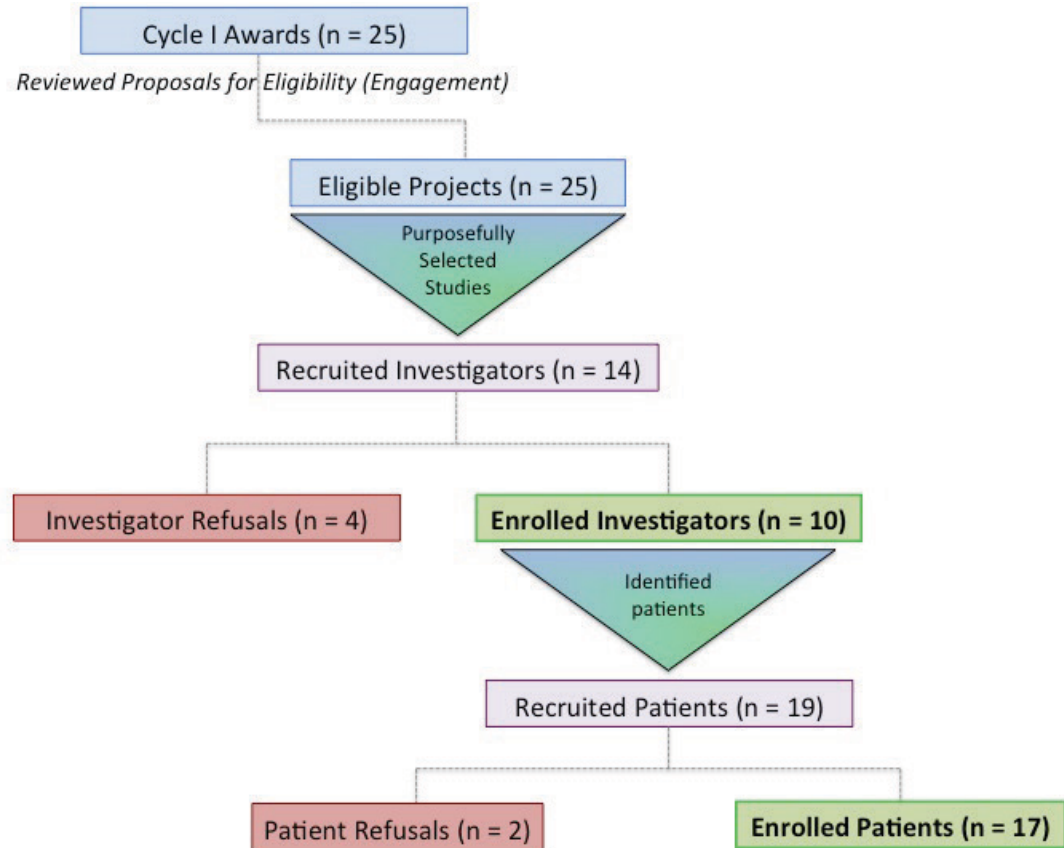


Figure 1.2: Recruitment Flowchart for Cycle I Awards



REFERENCES

- ATLAS.ti. (1999). (Version 7 ed.) [Computer Software]. Berlin: Scientific Software Development.
- Beatty, P. C., & Willis, G. B. (2007). Research synthesis: The practice of cognitive interviewing. *Public Opinion Quarterly*, 71(2), 287-311. doi:10.1093/poq/nfm006
- Boote, J., Telford, R., & Cooper, C. (2002). Consumer involvement in health research: A review and research agenda. *Health Policy (Amsterdam, Netherlands)*, 61(2), 213-236. doi:S0168851001002147
- Brett, J., Staniszevska, S., Mockford, C., Herron-Marx, S., Hughes, J., Tysall, C., & Suleman, R. (2014). Mapping the impact of patient and public involvement on health and social care research: A systematic review. *Health Expectations: An International Journal of Public Participation in Health Care and Health Policy*, 17(5), 637-650. doi:10.1111/j.1369-7625.2012.00795.x
- Caron-Flinterman, J. F., Broerse, J. E., & Bunders, J. F. (2005). The experiential knowledge of patients: A new resource for biomedical research? *Social Science & Medicine*, 60(11), 2575-2584. doi:S0277-9536(04)00570-2
- Concannon, T. W., Fuster, M., Saunders, T., Patel, K., Wong, J. B., Leslie, L. K., & Lau, J. (2014). A systematic review of stakeholder engagement in comparative effectiveness and patient-centered outcomes research. *Journal of General Internal Medicine*. doi:10.1007/s11606-014-2878-x
- Domecq, J. P., Prutsky, G., Elraiyah, T., Wang, Z., Nabhan, M., Shippee, N., . . . Murad, M. H. (2014). Patient engagement in research: A systematic review. *BMC Health Services Research*, 14, 89-6963-14-89. doi:10.1186/1472-6963-14-89
- Emanuel, E. J., Wendler, D., & Grady, C. (2000). What makes clinical research ethical? *Journal of the American Medical Association*, 283(20), 2701-2711. doi:jsc90374
- Forsythe, L. P., Szydlowski, V., Murad, M. H., Ip, S., Wang, Z., Elraiyah, T. A., . . . Hickam, D. H. (2014). A systematic review of approaches for engaging patients for research on rare diseases. *Journal of General Internal Medicine*, 29 Suppl 3, S788-800. doi:10.1007/s11606-014-2895-9
- Gradingier, F., Britten, N., Wyatt, K., Froggatt, K., Gibson, A., Jacoby, A., . . . Popay, J. (2013). Values associated with public involvement in health and social care research: A narrative review. *Health Expectations: An International Journal of Public Participation in Health Care and Health Policy*. doi:10.1111/hex.12158

Hanley, B., Bradburn, J., Barnes, M., Evans, C., Goodare, H., Kelson, M., . . . Wallcraft, J. (2003). *Involving the public in NHS, public health and social care research: Briefing notes for researchers* (2nd ed.). Eastleigh: INVOLVE Support Unit. Retrieved from [http://www.twocanassociates.co.uk/perch/resources/files/Briefing Note Final_dat\(1\).pdf](http://www.twocanassociates.co.uk/perch/resources/files/Briefing%20Note%20Final_dat(1).pdf)

Institute of Medicine Committee on Comparative Effectiveness Research Prioritization. (2009). *Initial national priorities for comparative effectiveness research*. Washington, DC: National Academies Press.

Israel, B. A., Schulz, A. J., Parker, E. A., Becker, A. B., Allen, A., & Guzman, J. R. (2003). Critical issues in developing and following community-based participatory research principles. In M. Minkler & N. Wallerstein (Eds.), *Community-Based Participatory Research for Health* (pp. 56-73). San Francisco, CA: Jossey-Bass.

Patient-Centered Outcomes Research Institute (PCORI). (2012a, June 18). PCORI announces Pilot Projects Program awards recipients, research topics. Retrieved from <http://www.pcori.org/content/pcori-announces-pilot-projects-program-awards-recipients-research-topics>

Patient-Centered Outcomes Research Institute (PCORI). (2012b, December 18). PCORI announces funding for first comparative effectiveness research projects. Retrieved from <http://www.pcori.org/content/pcori-announces-funding-first-comparative-effectiveness-research-projects>

Patient-Centered Outcomes Research Institute (PCORI). (2014, October 6). About us. Retrieved from <http://www.pcori.org/about-us>

Patient-Centered Outcomes Research Institute (PCORI). (2015). What we've funded. Retrieved from <http://www.pcori.org/research-results>

Patient Protection and Affordable Care Act of 2010, Pub. Law No. 111-148, 124 Stat 727, §6301.

Roehr, B. (2010). More stakeholder engagement is needed to improve quality of research, say US experts. *British Medical Journal*, 341(c4193). <http://dx.doi.org/10.1136/bmj.c4193>

Sandelowski, M. (2000). Focus on research methods: Whatever happened to qualitative description? *Research in Nursing and Health*, 23(4), 334-340. doi:10.1002/1098-240X(200008)23:4<334::AID-NUR9>3.0.CO;2-G

Selby, J. V., Beal, A. C., & Frank, L. (2012). The Patient-Centered Outcomes Research Institute (PCORI) national priorities for research and initial research agenda. *Journal of the American Medical Association*, 307(15), 1583-1584. doi:10.1001/jama.2012.500

- Shippee, N. D., Domecq Garces, J. P., Prutsky Lopez, G. J., Wang, Z., Elraiyah, T. A., Nabhan, M., . . . Murad, M. H. (2013). Patient and service user engagement in research: A systematic review and synthesized framework. *Health Expectations: An International Journal of Public Participation in Health Care and Health Policy*. doi:10.1111/hex.12090
- Snape, D., Kirkham, J., Preston, J., Popay, J., Britten, N., Collins, M., . . . Jacoby, A. (2014). Exploring areas of consensus and conflict around values underpinning public involvement in health and social care research: A modified Delphi study. *BMJ Open*, 4(1), e004217-2013-004217. doi:10.1136/bmjopen-2013-004217
- Sox, H. C., & Greenfield, S. (2009). Comparative effectiveness research: A report from the Institute of Medicine. *Annals of Internal Medicine*, 151(3), 203-205. doi:0000605-200908040-00125
- Staley, K. (2009). *Exploring impact: Public involvement in NHS, public health and social care research*. Eastleigh: INVOLVE. Retrieved from http://www.invo.org.uk/wp-content/uploads/2011/11/Involve_Exploring_Impactfinal28.10.09.pdf
- Staniszewska, S., Adebajo, A., Barber, R., Beresford, P., Brady, M., Brett, J., . . . Williamson, T. (2011). Developing the evidence base of patient and public involvement in health and social care research: The case for measuring impact. *International Journal of Consumer Studies*, 35(6), 628-632. doi:10.1111/j.1470-6431.2011.01020.x
- Tritter, J. Q., & McCallum, A. (2006). The snakes and ladders of user involvement: Moving beyond Arnstein. *Health Policy (Amsterdam, Netherlands)*, 76(2), 156-168. doi:S0168-8510(05)00131-4
- Washington, A. E., & Lipstein, S. H. (2011). The Patient-Centered Outcomes Research Institute--promoting better information, decisions, and health. *The New England Journal of Medicine*, 365(15), e31. doi:10.1056/NEJMp1109407
- Workman, T., Maurer, M., & Carman, K. (2013). Unresolved tensions in consumer engagement in CER: A US research perspective. *Journal of Comparative Effectiveness Research*, 2(2), 127-134. doi:10.2217/cer.13.6

MANUSCRIPT 2: Challenges to and Successful Strategies for Patient Engagement in Research

ABSTRACT

Despite increasing support for patient engagement in research, guidance on how to engage patients is limited. The aim of this study was to describe challenges to and successful strategies for patient engagement. Interviews were conducted with 19 investigators of projects funded by the Patient-Centered Outcomes Research Institute (PCORI) and with 33 patients engaged in 18 of the same 19 projects. Informants reported experiencing logistical and substantive challenges. Logistical challenges referred to challenges in planning engagement, including extra time and effort, difficulty working with investigators' institutions, and difficulty holding meetings. Substantive challenges included challenges to selecting, orienting, and interacting with patients and to incorporating patient feedback. Successful techniques included using existing resources, communicating goals, providing patient education, and treating patients respectfully. These findings suggest actions for consideration that are relevant to funders, institutions, and researchers. Modifications to policies, the development of programs and researcher networks, and the provision of resources and training are suggested. As opportunities for engagement grow, bolstering the infrastructure for engagement must remain a priority.

INTRODUCTION

Patient engagement—when researchers involve patients in research in ways other than as subjects of research—is gaining support. Strategies include having patients on advisory boards or as research team members, serving as advisors or partners (Hanley et al., 2003; Staley, 2009). A growing evidence base shows that patient engagement may enhance relevance to end users, improve recruitment, and produce higher quality research (Brett et al., 2014; Concannon et al., 2014; Domecq et al., 2014; Shippee et al., 2013). Because of these benefits, some funding organizations support engagement, while others require it. Most notably, the Patient-Centered Outcomes Research Institute (PCORI), a non-profit research funding organization Congressionally authorized under the Patient Protection and Affordable Care Act (2010), requires engagement in its funded comparative effectiveness research (CER) (PCORI, 2014a).

While a robust literature on community-based participatory research (CBPR) exists, including on successful strategies like developing reciprocal relationships (Viswanathan et al., 2004) and identifying common goals (Israel, Schulz, Parker, & Becker, 1998), and on challenges from power imbalances to time demands (Israel et al., 1998), guidance specifically on *patient engagement* is limited. Some literature exists on challenges to patient engagement including on lack of time and resources (Brett et al., 2014; Cargo & Mercer, 2008; Concannon et al., 2014; Domecq et al., 2014; Minkler, Blackwell, Thompson, & Tamir, 2003; Saunders & Girgis, 2010; Trivedi & Wykes, 2002) and on difficulty finding representative patients (Concannon et al., 2014; Snape et al., 2014; Stewart & Liabo, 2012), and some literature exists on successful strategies

(Hewlett et al., 2006; Hoffman, Montgomery, Aubry, & Tunis, 2010). However, most report on the experiences of individual investigators or are reviews of individual studies.

As funders increasingly support engagement, there will be more opportunities for researchers to engage patients. Such opportunities may attract investigators with little experience engaging patients as well as those more experienced. Having a better understanding of successful engagement strategies, challenges, and ways to address challenges is a priority.

Investigators funded under PCORI's early initiatives have navigated patient engagement in an environment of limited guidance and may be considered trailblazers from which we can learn, informing engagement strategies and recommendations for funders, institutions, and researchers going forward. The research questions of this qualitative study were: (i) "What were the challenges faced by researchers and patients when participating in patient engagement work in the first set of PCORI-funded projects?" and (ii) "What strategies did researchers and patients identify as helpful or successful for patient engagement?"

METHODS

This study's aim was to understand challenges to and successful strategies for patient engagement through qualitative interviews with PCORI-funded principal investigators (PIs) and patients engaged in their projects. Approval by the Johns Hopkins Bloomberg School of Public Health Institutional Review Board (JHSPH IRB) was obtained for this study.

Sampling

Purposeful sampling was used to select projects from PCORI's earliest funding awards: the 50 Pilot Projects (two-year projects awarded in June 2012) (PCORI, 2012a) and the 25 Cycle I Awards (three-year projects awarded in December 2012) (PCORI, 2012b). These were chosen because they were furthest underway when interviews were conducted (December 2013 through May 2014).

The goal of sampling was to capture projects using different engagement approaches (e.g., advisory groups, focus groups/interviews, surveys, patient research team members) while also ensuring diversity with respect to other attributes: the patient population engaged (e.g., healthy patients, sick patients, patients from advocacy groups, patients from minority populations), whether non-patient stakeholders were also engaged (e.g., policymakers, providers, purchasers), the research topic (e.g., chronic or rare diseases, behavioral health), and whether patients and researchers had prior relationships. Funding applications were reviewed to identify studies with patient engagement—defined as having patients, patient representatives, community members, and/or caregivers involved in ways other than as research subjects—as only these studies were eligible. Eligible projects were grouped by engagement approach, and projects were selected to ensure each approach was included and that variation was achieved regarding the aspects described above. The principal investigators of the selected eligible projects were contacted by email and/or phone about their interest in participating in an interview for this study and their willingness to refer patients engaged in their projects for potential interviews. Interested investigators were enrolled in the study.

Participating investigators identified one to two patients engaged in their projects who were somewhat different from each other (e.g., age, gender, health, relationship with advocacy groups, experience with engagement) to approach for participation in an interview. Investigators obtained patients' permission before sharing their contact information.

Interviews and Questionnaires

Interviews were audio-recorded and oral consent was obtained. During recruitment and informed consent, participants were told that the interviewer (LE) worked at PCORI and that PCORI was aware of but had no involvement in the project. Participants were informed that the project comprised the research for the student's doctoral dissertation, and that PCORI would have no access to information about which studies/individuals were recruited or interviewed, nor would PCORI have access to any data collected.

Participants completed a questionnaire on demographic and background information. Prior to conducting study interviews, two cognitive interviews were conducted to test the understandability of interview questions. One was with a researcher with experience engaging patients and the other was with a patient on a research advisory board. The interview guide and questionnaire were also reviewed by a community research advisory council, and modifications were made in response to feedback.

Analysis

Data collection and analysis occurred iteratively with interviews conducted until informational redundancy was reached. Audio-recordings were transcribed and reviewed

for accuracy. The coding scheme was developed deductively from interview questions and inductively from open coding. Codes were applied to all transcripts. A second coder coded six transcripts to assess reliability; a high concordance between coders (80% agreement) was obtained. The software ATLAS.ti (1999) was used during analysis.

RESULTS

This section reports study findings, including demographic and background information about participants obtained from the questionnaire, a brief description of engagement approaches used by projects, and then key findings related to (i) logistical challenges to patient engagement, (ii) substantive challenges to patient engagement, and (iii) successful strategies for patient engagement. Interview quotations used in this section are labeled by random study identification and by respondent (“R” for researcher; “Pt1” or “Pt2” for the first or second patient interviewed from a study).

Participants

There were 67 studies with engagement and thus eligible. A total of 33 PIs were recruited from eligible projects based on the sampling criteria described above; 19 PIs participated and 14 declined. Of those that declined, two did not respond after four attempts and the others cited lack of time (n=4), IRB concerns (n=1), not yet engaging patients (n=1), not being comfortable having patients interviewed (n=4), and no longer being able to contact patients whom they engaged (n=2). The proportion of declining investigators was higher among Pilot Projects. Interviews were conducted with a total of 19 PIs from 19 projects, 11 in person and 8 by phone. The average interview was 45 minutes (range: 27 to 67 minutes). Most PIs were white, and about half were female, had

served as PI on seven or more grants including their PCORI study, and had experience engaging patients.

A total of 37 patients were approached for interviews; 33 participated and four declined. One declining patient did not respond after four attempts, one cited lack of time, and two cited lack of interest. A total of 33 patients (from 18 of the same 19 projects) were interviewed, 14 in person and 19 by phone. The average interview was 42 minutes (range: 16 to 62 minutes). Most patients were white, and over two-thirds were female, employed, and at least college educated. About half reported having a chronic condition, and more than a third had previously engaged in research. Recruitment numbers and participant characteristics are summarized in Table 2.1 and Table 2.2, respectively.

Engagement Approaches

Most projects used multiple engagement approaches (n=14), including involving patients in advisory groups (n=16), as co-investigators or research team members (n=9), and in focus groups/interviews (n=7) or surveys (n=3). Advisory groups usually met at least quarterly to discuss study progress and provide input. The nine studies engaging patients as co-investigators or team members involved patients in regular meetings to get their input, and over half of these studies (n=5) had patients leading other engagement activities (e.g., patient advisory groups). Focus groups, interviews, and surveys usually involved single meetings to get feedback on particular areas (e.g., outcomes, instruments). Nearly half of studies (n=9) engaged patients in pilot testing study materials, and over half (n=11) had preexisting researcher-patient relationships.

Challenges

When asked what hindered engagement in the project, participants reported logistical and substantive challenges. Logistical challenges referred to challenges in planning engagement, including extra time and effort, working with investigators' institutions, and holding meetings. Substantive challenges included challenges to selecting, orienting, and interacting with patients and to incorporating patient feedback. While researchers and patients articulated the same challenges, researchers discussed logistical challenges and resolutions more than patients. Challenges are described below, and select quotations are provided in Table 2.3.

Patterns between funding cycles and among engagement approaches were explored and no differences regarding the nature or frequency of challenges were found. Because patterns were not noted, data are not disaggregated by funding cycle or engagement approach in the presentation of findings on challenges and successful strategies..

Logistical Challenges

Extra Time and Work

Over half of investigators (n=11) noted the extra time or work needed to build capacity and organize engagement. This preparation was necessary as failing to do so could slow the research process. About a quarter of investigators (n=5) failed to anticipate the extent of work: "It really takes time to set up this process and to do it well... it can delay you" (108R). One investigator described the work required to incorporate patient feedback *after* engagement: "You're on a fixed timeline and a fixed

budget... that's been challenging" (107R). A few investigators (n=3) noted that "lessons learned would be to anticipate [the time required] and work it into the timeline" (109R).

Institutional Processes

About half of investigators (n=9) faced institutional barriers to actively engaging patients. Arranging patient partners' compensation could be administratively onerous: "Just invoicing everybody for their time and doing all the paperwork to pay them for each of the calls takes time, and I would say we underestimated that" (113R). One investigator expressed frustration that her institution capped compensation to patients, who sometimes missed work or needed childcare to participate.

About a third of researchers (n=6) reported difficulty with institutional review boards (IRBs). IRBs did not understand that patient partners were not research subjects needing to sign consent forms. Communication with the IRB helped address this. There were also challenges with completing IRB-required human subjects protections training. An investigator said it required "a huge amount of resource," and patients from that study agreed, one calling it "one of the toughest hurdles" (115R, 115Pt1).

Scheduling and Having Meetings

Difficulty having meetings was reported by about a quarter of investigators (n=5). To address this, one investigator scheduled meetings when stakeholders were already at the institution; another held three meetings throughout the week when no time worked for everyone.

A few patients (n=5) reported difficulty meeting in-person because of physical impediments or transportation issues. Two investigators responded by communicating

electronically and meeting virtually. While high technology strategies were generally successful for projects employing them, they had drawbacks. Some patients had trouble accessing or using the Internet. One researcher mailed materials to an elderly patient with no Internet access; two investigators switched from virtual to in-person meetings in response to patients' preferences.

Substantive Challenges

Selecting Patients

Investigators and patients described challenges to the process of selecting patients for engagement. About half of investigators (n=9) expressed concern that interested patients did not fully represent the patient population to which the study applied. One investigator addressed this by recruiting from several segments of the population to include healthy, ill, at-risk, low-income, and higher income patients. Alternatively, two investigators prioritized patients with experience and investment in the topic over representative patients.

Investigators generally spoke highly of patient partners; however, about a fifth (n=4) wished they had done more screening: "I would probably figure out a way to recruit patients more like a job interview... I would invest a lot more time in recruiting and selecting patients that would fit with the team" (115R).

A third of patients (n=11) articulated the importance of screening: "If you are getting a patient that has not been... screened or any due diligence done, you don't know what you're getting, and if you're going to get somebody who is just coming in to complain about their lot in life, it is not going to help a study" (116Pt2). While

researchers who discussed screening emphasized selecting patients that fit with the team, patients emphasized selecting patients who could focus on the patient population's needs rather than personal issues.

Over a third of patients (n=13) described the ideal type of patient to engage. Of this group, about half (n=6) felt that trained advocates provided contextualized input that regular patients could not while the other half (n=7) felt that regular patients with no medical background provided a needed outside perspective. A few patients (n=4) saw equal but different value in both, suggesting regular patients were ideal sounding boards while advocates were ideal for setting agendas.

Orienting Patients

It could be difficult and time consuming for researchers to explain research concepts and orient patients. About half of investigators (n=9) and about a third of patients (n=12) noted that if time were not devoted to teaching patients about research methods and study topics, patients' lack of familiarity could delay meetings, impede their contributions, and make them uncomfortable. Patients needed courage to ask questions: "I have got to have the guts to ask: 'Can I just ask a question?... Can you just explain to me what you just said in terms that I can understand?'" (102Pt1).

Few resolutions were reported. One investigator, as described by a patient, told patients: "Why don't we have a pre-meeting before the meeting?... Come with all of your questions, your acronyms, what you understand, what you want further clarification on" (115Pt1).

The Engagement Interaction Itself

Investigators described feeling unprepared for engaging patients, and patients described challenges they faced during engagement. About a third of investigators (n=6), mostly without engagement experience, reported not fully grasping what engagement entailed or how to do it well and struggled to find appropriate activities, formats, or questions to ask patients. One investigator found that asking targeted questions yielded better feedback than did asking for general comments. About a quarter of researchers (n=5), both with and without prior experience, expressed wanting training, with two specifically noting disparities between researcher and patient training: the institution “spends a half day conditioning [the patient] or teaching [the patient] how to interact with the researchers, but there's no effort to condition the researchers” (102R).

About a fourth of patients (n=8) reported feeling frustrated when researchers used jargon because it impeded their participation: meetings were “all research stuff; it was all these acronyms... that really didn't have a lot of meaning” (117Pt1). A quarter of investigators (n=5) addressed this by pausing to explain jargon.

Incorporating Patient Feedback

Investigators described two kinds of challenges to incorporating patient feedback. The first, reported by about a quarter of investigators (n=5), occurred when feedback could not be implemented because of lack of time/resources or feasibility. For example, patients from one study wanted the study available to non-English speakers, but researchers lacked resources to translate materials into other languages.

The second challenge, also reported by about a quarter of investigators (n=5), occurred when feedback was scientifically inappropriate. As one researcher explained, “They don’t have a scientific understanding of research design, so they may want you to do stuff that... might actually compromise, especially, the internal validity” (119R). This occurred when patients recommended substantive changes to study designs or validated instruments.

About a third of investigators (n=7) reported difficulty sharing control of the research with patients. While challenges to incorporating patient feedback could not be resolved, about a third of investigators (n=6) found it helpful to explain why feedback was not used.

Successful Strategies

Informants were asked what worked well for engagement; responses included using existing resources, communicating goals, providing patient education, and treating patients respectfully.

Using Existing Resources

About a third of researchers (n=6) took advantage of established advisory councils to find patients to engage. A benefit of this was patients’ familiarity with clinical practice/research. A patient described it as “wonderful for us because it has been a way to have people who are trained” (101Pt1).

Communicating Clear Goals

About a third of investigators (n=7) and nearly a fifth of patients (n=6) described the importance of knowing and communicating goals. As one researcher said: “You just have to be so clear why you're doing this, to yourself and to the people that are participating” (113R). About a fourth of patients (n=8) reported appreciating receiving agendas before meetings to inform them about plans or receiving summaries after meetings to recap what was accomplished.

Providing Patient Education

Although patient education was challenging, it was also regarded as crucial. About a quarter of investigators (n=5) described helpful techniques including developing plain language information sheets, sharing articles, and presenting on key topics. One investigator created a glossary, which the patient for whom it was created found essential: “I need to understand those medical terms because I don't have a medical degree” (102Pt1). Another investigator designed a training in which a lecture for clinicians was adapted for patients. This method’s success, in prior work and in the project, reinforced the investigator’s belief that anyone can become proficient in advanced topics when information is provided appropriately. A patient from that study viewed the training as enabling patients’ participation: it put “everybody on a more equal plane... so that we all shared the same language and can speak about it in a way that was medically relevant” (104Pt2).

Treating Patients Respectfully

Over half of researchers (n=10) and nearly two-thirds of patients (n=21) described at least one of two ways in which it was helpful for interactions to be conducted with respect. One way occurred when researchers acted politely toward patients; the other occurred when researchers respected patient input.

First, about a fifth of researchers (n=4) and a fourth of patients (n=9) described ways in which researchers acted politely, making patients feel more able to participate. This included researchers taking care of paperwork for patient compensation, accommodating schedules, holding meetings at convenient locations, and building rapport. As one patient expressed: “They feed us and they make sure we’re all comfortable and they engage us on a personal level” (103Pt2).

Second, over half of both researchers (n=10) and patients (n=17) described the importance of researchers respecting patient input. This included researchers soliciting and valuing patient opinions, promoting equality, and sharing how feedback was used. Patients reported feeling respected when researchers valued their perspectives: “[PI] has done a really good job of checking in all the time and saying, ‘What’s working? What’s not working?,’ and addressing what’s not working right away” (114Pt1). Researchers expressed the same view: “Being respectful of their expertise is enormous” (104R).

Nearly half of researchers (n=9) reported promoting equality. About a fifth of investigators (n=4) used first names instead of professional titles. One researcher had stakeholders develop guiding principles to “enforce the fact that everybody’s voice was equal” (102R). One researcher described the importance of elevating patients to leadership roles including as co-investigators: “Doing things behaviorally that

demonstrate that you are behind some level of equality in power here, in this situation, is very, very important” (101R). A few patients (n=5) reported feeling respected by being involved in decision-making.

Nearly a third of researchers (n=6) described the importance of reporting back how patient feedback was or was not used: “If you consider something and decide not to respond to it: ‘We didn't do that earlier and here’s the reason why.’ So... really just do things that make them feel like you are treating them respectfully” (112R).

DISCUSSION

This study sought to understand what worked well for engaging patients, what was challenging, and resolutions to challenges from a sample of PCORI projects.

Successful techniques included communicating goals, engaging patients from existing councils, educating patients, and treating patients respectfully. The fact that engaging patients from existing councils was successful suggests engaging patients with more experience is helpful, perhaps by allowing for meaningful discussions to take place sooner or by lessening demands on researchers to educate patients. In fact, patient education, though a successful strategy, was challenging and required skill and planning. Some successful techniques reported here have been described in the literature, lending further credence to these findings. For example, one group developed a framework that includes training, communicating patient roles, and treating patients respectfully (Hewlett et al., 2006). Others developed principles to guide stakeholder engagement in CER and specified strategies like preparatory meetings with patients (Hoffman et al., 2010).

Investigators with varying experience faced logistical challenges including extra time and work, institutional barriers, and difficulties with meeting logistics. Creative solutions resolved challenges to meetings, with a few researchers accommodating patient preferences for meeting modality. Trade-offs of virtual versus in-person engagement have been discussed in the literature, as have hybrid approaches capitalizing on the benefits of both (Lavallee, Wicks, Alfonso Cristancho, & Mullins, 2014). Some practical challenges reported here are in the literature including limited resources and time (Brett et al., 2014; Cargo & Mercer, 2008; Concannon et al., 2014; Minkler et al., 2003; Trivedi & Wykes, 2002) and IRB challenges (Cargo & Mercer, 2008; Concannon et al., 2014); however, consistent with our findings, resolutions remain limited.

Participants reported substantive challenges regarding how to select, orient, and interact with patients. Challenges to incorporating patient feedback have been described in the literature (Buchanan, Miller, & Wallerstein, 2007; Trivedi & Wykes, 2002). While informants did not offer strategies to address this, explaining why feedback was not used was helpful. Consistent with previous literature, researchers expressed concerns about representativeness (Concannon et al., 2014; Snape et al., 2014; Stewart & Liabo, 2012). Informants endorsed screening patients to improve the selection process.

Implications for Policy

While informants experienced substantive and logistical challenges, some also found resolutions to challenges or reported successful strategies in these areas. This indicates that additional guidance and resources may be valuable. A series of actions may help address challenges identified by our informants and promote successful strategies. These actions for consideration pertain to policies, programs, training,

resources, and network development and, as such, are relevant to funders, institutions, and researchers.

Given the success of engaging patients from existing councils, researchers could benefit from being matched with experienced patients. PCORI's Ambassador Program, a relatively new initiative, is one such program that serves to "connect patients and other stakeholders with common interests and help position them as potential partners" (PCORI, 2013). While matching programs alone are unlikely to improve representativeness, coupled with patient outreach and training, they can streamline patient selection and facilitate engaging patients from diverse backgrounds. Engaging experienced and inexperienced patients together can also help prepare a new cadre of patient research partners.

Challenges from patients' lack of familiarity with research methods and study topics suggest the need for targeted training in these areas. Layperson training on research methods and ethics, like what exists for CBPR (Allen, Culhane-Pera, Call, & Pergament, 2010; Anderson, 2012), could be adapted. Equally important is researcher training on engagement. Funders could sponsor trainings and produce more guidance on successful strategies, and experienced researchers could train inexperienced researchers. PCORI's Patient and Family Engagement Rubric, which was published after studies in this sample were funded, is one example (PCORI, 2014b). More resources of this nature can be developed and shared to promote collaborative learning.

Researchers may act collectively to resolve challenges more efficiently. Researchers could identify how this might best occur; possibilities include researchers

participating in networks created by funders, being connected on an ad hoc basis by project officers, using web-based platforms, or forming mentorships.

Finally, to address institutional barriers, model policies on compensation for patients engaged in research and on IRB classification of patient partners could serve as templates for research institutions, reducing cumbersome processes and inappropriate classification of engagement partners as research subjects.

Limitations

There were several limitations to this study. One is that it only included PCORI projects, which are unique given their support for engagement. While this could limit generalizability, the fact that challenges were identified even among well-resourced projects and the consistency between our findings and the literature suggest findings are relevant more broadly. Another limitation is that projects were funded early in PCORI's lifespan and may differ from later projects. These researchers may have had more experience with patient engagement than later PCORI researchers. Alternatively, they may have had less familiarity with successful strategies since some resources now available were not when studies began. Nonetheless, experiences from early initiatives will resonate with the many investigators and patients new to engagement. A third limitation is that those who declined participation may have had different experiences that were not captured. Declining investigators were more senior so they may have had more experience; however, investigators with and without experience reported challenges in this study. Last, this study included a subset of projects; other methods could accommodate collecting information from a larger set of projects, although would miss the rich descriptions reported here.

CONCLUSION

This study identified successful techniques for engagement and challenges impeding engagement. Modifications to policies, the development of programs and researcher networks, and the provision of resources and training are steps that could mitigate challenges. Future research should evaluate the effectiveness of such actions in strengthening engagement. As opportunities for experienced and inexperienced researchers grow, bolstering the infrastructure for successful engagement must remain a priority.

TABLES

Table 2.1: Overview of Recruitment and Enrollment Numbers						
	Pilot Projects		Cycle I Awards		TOTAL	
	PIs	Patients	PIs	Patients	PIs	Patients
Eligible	42	n/a	25	n/a	67	n/a
Recruited	19	18	14	19	33	37
Refused	10	2	4	2	14	4
Interviewed	9	16	10	17	19	33

This table reports the number that were eligible, recruited, refused participation, and participated for researchers and patients by funding cycle and in total. The total number of patients engaged in eligible projects was not available, indicated by “n/a”.

Table 2.2: Characteristics of Study Participants		
	PIs (n=19)	Patients (n=33)
Age		
<i>Under 30 years</i>	1	3
<i>31-40 years</i>	4	1
<i>41-50 years</i>	5	4
<i>51+ years</i>	9	25
Race		
<i>African American (non-Hispanic)</i>	1	4
<i>Asian/Pacific Islander (non-Hispanic)</i>	1	1
<i>White (non-Hispanic)</i>	17	26
<i>Hispanic</i>	0	2
Gender		
<i>Female</i>	10	23
<i>Male</i>	9	10
Additional Patient Demographics		
<i>Prior experience being engaged in research</i>	-	13
<i>College or graduate degree</i>	-	25
<i>Employed full or part time</i>	-	24
<i>Self-reported chronic disease/condition</i>	-	17
Additional Researcher Demographics		
<i>Prior experience engaging patients in research</i>	10	-
<i>Served as PI on ≤ 6 grants (inclusive)</i>	10	-
<i>Served as PI on ≥ 7 grants (inclusive)</i>	9	-
<i>Served on academic faculty ≤ 10 years</i>	8	-
<i>Served on academic faculty ≥ 10 years</i>	11	-

This table reports data obtained from a questionnaire administered to interview participants. All data are self-reported. Some questions were only asked of researchers and some were only asked of patients, indicated by “-” in cases where there are no data to report.

Table 2.3: Select Quotations from Interviews	
Logistical Challenges	
Extra Time and Work:	“It’s much easier... just to drive it forward. When you have to backup a little bit and get input and be participatory, it’s time consuming, and I would suspect that many researchers would be challenged by this model.” [118Pt1]
Institutional Processes:	“Just the logistics...it is a challenge, like institutionally, how do you pay these folks, those kinds of things. And you know, the IRB issues, they’re just red tape, extra red tape.” [107R]
Meetings:	“There are things I think everyone faces – logistical challenges... Physically meeting is hard. Some folks are disabled now. Some people work and have lives. And it’s just hard... any meeting with more than 2 people, it’s really hard to orchestrate.” [117R]
Substantive Challenges	
Selecting Patients:	“It can be a detriment in the sense that if people need to talk more than listen, they may not be ready to be an active participant in a way that’s helpful... I’ve seen that a lot where somebody just said, ‘I know a neighbor who had this experience and she’d be a good person to come be on an advisory committee.’ Well, sometimes yes, sometimes no. I think that people who have used their experience to understand the larger experience of other people involved in your care can be very helpful.” [114Pt2]
Orienting Patients:	“When you involve non-professional science people in a science team, you’re going to have a lot of time taken out of a meeting to discuss something that... we don’t understand.” [115Pt2]
The Engagement Interaction Itself:	“It feels very fragmented because we don’t meet very often. Sometimes it’s easy to lose the thread of what’s going on... so that makes it a little harder, I think, to stay focused.” [103Pt2]
Incorporating Patient Feedback:	“There was a suggestion that we have this survey available in other languages and so we examined that and it would’ve been good... But it’s hard, and there’s a limit to the money and all of that.” [103Pt1]
Successful Strategies	
Using Existing Resources:	“We got help from the head of the [patient resource center at PI’s institution] to identify some patients and family representatives who already were serving on these kinds of committees and were interested in participating.” [112R]
Communicating Clear Goals:	“Just making clear what the expectations are, of their role, and giving them feedback periodically and updates... so that they don’t feel like their review or feedback was for naught.” [111R]
Providing Patient Education:	“When you bring in stakeholders that are not physician slash researchers, whether they are a patient or not a patient, you have to get people to a certain level in order to be able to interact. There has to be common understanding to get people to a certain level, so things like developing a glossary, right? It’s time consuming, but it’s really essential to do in educating and making sure that people can actively contribute to conversations about research.” [102R]
Treating Patients Respectfully:	“I think that just the basic respect for each other’s expertise and understanding that our community members are experts in their community; and they bring that to the table, to us. And we are experts in research design and research, and those types of things, and we bring that to the table. We each bring something different, but it’s equally as important.” [105R]

REFERENCES

- Allen, M. L., Culhane-Pera, K., Call, K. T. & Pergament, S. (2010). Partners in research: Curricula to prepare community and faculty for CBPR partnerships. CES4Health.info. Retrieved from <http://www.healthdisparities.umn.edu/community-engagement/cbpr-collaborations/>
- Anderson, E. E. (2012). Community involvement in research training: Facilitator manual & participant workbook. Retrieved from <http://www.ccts.uic.edu/repository/cirtification>
- ATLAS.ti. (1999). (Version 7 ed.) [Computer Software]. Berlin: Scientific Software Development.
- Brett, J., Staniszewska, S., Mockford, C., Herron-Marx, S., Hughes, J., Tysall, C., & Suleman, R. (2014). Mapping the impact of patient and public involvement on health and social care research: A systematic review. *Health Expectations: An International Journal of Public Participation in Health Care and Health Policy*, 17(5), 637-650. doi:10.1111/j.1369-7625.2012.00795.x
- Buchanan, D. R., Miller, F. G., & Wallerstein, N. (2007). Ethical issues in community-based participatory research: Balancing rigorous research with community participation in community intervention studies. *Progress in Community Health Partnerships: Research, Education, and Action*, 1(2), 153-160. doi:10.1353/cpr.2007.0006
- Cargo, M., & Mercer, S. L. (2008). The value and challenges of participatory research: Strengthening its practice. *Annual Review of Public Health*, 29, 325-350. doi:10.1146/annurev.publhealth.29.091307.083824
- Concannon, T. W., Fuster, M., Saunders, T., Patel, K., Wong, J. B., Leslie, L. K., & Lau, J. (2014). A systematic review of stakeholder engagement in comparative effectiveness and patient-centered outcomes research. *Journal of General Internal Medicine*. doi:10.1007/s11606-014-2878-x
- Domecq, J. P., Prutsky, G., Elraiyyah, T., Wang, Z., Nabhan, M., Shippee, N., . . . Murad, M. H. (2014). Patient engagement in research: A systematic review. *BMC Health Services Research*, 14, 89-6963-14-89. doi:10.1186/1472-6963-14-89
- Hanley, B., Bradburn, J., Barnes, M., Evans, C., Goodare, H., Kelson, M., . . . Wallcraft, J. (2003). *Involving the public in NHS, public health and social care research: Briefing notes for researchers* (2nd ed.). Eastleigh: INVOLVE Support Unit. Retrieved from [http://www.twocanassociates.co.uk/perch/resources/files/Briefing Note Final_dat\(1\).pdf](http://www.twocanassociates.co.uk/perch/resources/files/Briefing%20Note%20Final_dat(1).pdf)

- Hewlett, S., Wit, M., Richards, P., Quest, E., Hughes, R., Heiberg, T., & Kirwan, J. (2006). Patients and professionals as research partners: Challenges, practicalities, and benefits. *Arthritis and Rheumatism*, 55(4), 676-680. doi:10.1002/art.22091
- Hoffman, A., Montgomery, R., Aubry, W., & Tunis, S. R. (2010). How best to engage patients, doctors, and other stakeholders in designing comparative effectiveness studies. *Health Affairs (Project Hope)*, 29(10), 1834-1841. doi:10.1377/hlthaff.2010.0675
- Israel, B. A., Schulz, A. J., Parker, E. A., & Becker, A. B. (1998). Review of community-based research: Assessing partnership approaches to improve public health. *Annual Review of Public Health*, 19, 173-202. doi:10.1146/annurev.publhealth.19.1.173
- Lavallee, D. C., Wicks, P., Alfonso Cristancho, R., & Mullins, C. D. (2014). Stakeholder engagement in patient-centered outcomes research: High-touch or high-tech? *Expert Review of Pharmacoeconomics & Outcomes Research*, 14(3), 335-344. doi:10.1586/14737167.2014.901890
- Minkler, M., Blackwell, A. G., Thompson, M., & Tamir, H. (2003). Community-based participatory research: Implications for public health funding. *American Journal of Public Health*, 93(8), 1210-1213. doi:10.2105/AJPH.93.8.1210
- Patient-Centered Outcomes Research Institute (PCORI). (2012a, June 18). PCORI announces Pilot Projects Program awards recipients, research topics. Retrieved from <http://www.pcori.org/content/pcori-announces-pilot-projects-program-awards-recipients-research-topics>
- Patient-Centered Outcomes Research Institute (PCORI). (2012b, December 18). PCORI announces funding for first comparative effectiveness research projects. Retrieved from <http://www.pcori.org/content/pcori-announces-funding-first-comparative-effectiveness-research-projects>
- Patient-Centered Outcomes Research Institute (PCORI). (2013, October 23). Ambassador Program overview. Retrieved from <http://www.pcori.org/sites/default/files/PCORI-Ambassador-Program-Overview.pdf>
- Patient-Centered Outcomes Research Institute (PCORI). (2014a, October 6). About us. Retrieved from <http://www.pcori.org/about-us>
- Patient-Centered Outcomes Research Institute (PCORI). (2014b, August 6). PCORI engagement rubric with table. Retrieved from <http://www.pcori.org/assets/2014/08/PCORI-Engagement-Rubric-with-Table.pdf>
- Patient Protection and Affordable Care Act of 2010, Pub. Law No. 111-148, 124 Stat 727, §6301.

- Saunders, C., & Girgis, A. (2010). Status, challenges and facilitators of consumer involvement in Australian health and medical research. *Health Research Policy and Systems/BioMed Central*, 8, 34-4505-8-34. doi:10.1186/1478-4505-8-34
- Shippee, N. D., Domecq Garces, J. P., Prutsky Lopez, G. J., Wang, Z., Elraiyah, T. A., Nabhan, M., . . . Murad, M. H. (2013). Patient and service user engagement in research: A systematic review and synthesized framework. *Health Expectations: An International Journal of Public Participation in Health Care and Health Policy*. doi:10.1111/hex.12090
- Snape, D., Kirkham, J., Preston, J., Popay, J., Britten, N., Collins, M., . . . Jacoby, A. (2014). Exploring areas of consensus and conflict around values underpinning public involvement in health and social care research: A modified Delphi study. *BMJ Open*, 4(1), e004217-2013-004217. doi:10.1136/bmjopen-2013-004217
- Staley, K. (2009). *Exploring impact: Public involvement in NHS, public health and social care research*. Eastleigh: INVOLVE. Retrieved from http://www.invo.org.uk/wp-content/uploads/2011/11/Involve_Exploring_Impactfinal28.10.09.pdf
- Stewart, R., & Liabo, K. (2012). Involvement in research without compromising research quality. *Journal of Health Services Research & Policy*, 17(4), 248-251. doi:10.1258/jhsrp.2012.011086
- Trivedi, P., & Wykes, T. (2002). From passive subjects to equal partners: Qualitative review of user involvement in research. *The British Journal of Psychiatry: The Journal of Mental Science*, 181, 468-472. doi:10.1192/bjp.181.6.468
- Viswanathan, M., Ammerman, A., Eng, E., Garlehner, G., Lohr, K. N., Griffith, D., . . . Whitener, L. (2004). Summary, Evidence Report/Technology Assessment No. 99: Community-based participatory research: Assessing the evidence. Rockville, MD: Agency for Healthcare Research and Quality.

MANUSCRIPT 3: When and Why is Patient Engagement in Research

Morally Important?

ABSTRACT

Patient engagement in research refers to researchers involving patients in research in ways other than as subjects of research. While there is increasing support for patient engagement, with justifications often based on its presumed effects, none of the rationales provided for engaging patients in research have been explored from a moral perspective. In light of the increased interest in patient engagement, it becomes important to explore the moral arguments that may motivate researchers to engage patients in their research or that may motivate funders to support such engagement. Drawing on the existing literature and on findings from empirical interviews with researchers and patients, this paper explicates arguments for the moral importance of patient engagement. This paper considers patient engagement as morally important both as a strategy to achieve morally important ends and also as an activity that itself is intrinsically morally valuable. Engaging patients in research has instrumental value because it serves as a strategy to enhance the relevance of the research to patients, to hold researchers accountable for decisions made in the context of research, and to help researchers learn specific ways to treat study participants with respect—all of which, I argue, are morally important for research. Further, patient engagement has non-instrumental value because certain patient engagement activities—namely, seeking patients out and listening to them—are constitutive of researchers' duty to respect the patient population as people with unconditional moral worth. In ascertaining the circumstances in which patient engagement is morally important, this analysis offers insights into the kinds of research in which there are compelling reasons for researchers to engage patients. Researchers, funders, and institutional review boards may consider policies endorsing patient engagement in research meeting certain conditions in accordance with the moral rationales explored in this analysis.

INTRODUCTION

Patient engagement in research refers to researchers involving patients in research in ways other than as subjects of research (Hanley et al., 2003). Patient engagement includes a range of activities involving patients as research partners at any stage of the research process. Researchers conducting a study comparing treatment options, for example, may consult with patients for selecting study outcomes or work with patients in designing the study and developing materials. Researchers may engage patients at a single point in time or they may interact with patients multiple times throughout the research process. Frameworks for patient engagement specify hierarchical levels of patient involvement in research decision-making (Boote, Telford, & Cooper, 2002; Charles & DeMaio, 1993; Hanley et al., 2003; Tritter & McCallum, 2006). At the bottom of the hierarchy, researchers solicit patient opinions on aspects of research but may or may not follow them (*consultation*); whereas, higher levels of engagement constitute ongoing partnerships in which researchers and patients share decision-making (*collaboration*) or, at the highest level, research is initiated and directed by patients (*user/consumer control*) (Boote et al., 2002; Hanley et al., 2003).

Patient engagement in research is increasingly gaining support. There have been calls for more engagement of patients and other health care stakeholders in all aspects of the research process (Sox & Greenfield, 2009; Upton & DeGette, 2015). The United States Congress authorized the Patient-Centered Outcomes Research Institute (PCORI) under the Patient Protection and Affordable Care Act of 2010 to fund comparative effectiveness research that is required to engage patients and other stakeholders in the research process (PCORI, 2014). PCORI's requirement suggests that patient engagement

has value specifically for comparative effectiveness research, research that compares the benefits and harms of alternative methods to treat clinical conditions or improve the delivery of care in the real world setting (Federal Coordinating Council for Comparative Effectiveness Research, 2009). The United Kingdom's National Institute for Health Research (NIHR) also requires active involvement of the public in a wide range of health research it funds (NIHR, 2014a), which suggests that patient engagement has value for other kinds of research in addition to comparative effectiveness research.

Explicit justifications for patient engagement in research are not always provided; however, when they are, they often appeal to the presumed effects of engagement. Emerging evidence suggests patient engagement can have practical benefits for the research process including enhancing the relevance of the research to patients (Ellis & Kass, forthcoming; Brett et al., 2014; Caron-Flinterman, Broerse, & Bunders, 2005; Concannon et al., 2014; Domecq et al., 2014; Shippee et al., 2013), and both PCORI (Selby, Beal, & Frank, 2012) and NIHR (2014b) cite enhanced relevance of the research to patients as a main reason for engaging patients. The belief that patient engagement can enhance researchers' accountability for decisions made within the context of research has also been cited as a rationale for patient engagement (Tritter & McCallum, 2006; Bastian, 1994; Bastian, 1998; Boote et al., 2002), and there have been reports that patient engagement can help researchers to learn specific ways to treat study participants with respect (Staley, 2009).

In light of the current research milieu in which patient engagement is increasingly becoming an expectation for various kinds of research, we are faced with questions of whether, when, and why patient engagement in research is morally important. This paper

aims to answer these questions by analyzing reasons that might motivate the moral importance of patient engagement. The moral importance of patient engagement could stem entirely from its instrumental contributions to the presumed ends of engagement or it could stem, in part, too, from some intrinsically important duty that is fulfilled by virtue of engaging patients. In this paper, I first explore how patient engagement can be said to contribute instrumentally to certain ends—namely, relevance, accountability, and respect—and the extent to which these ends are morally important for research. After arguing that these ends of patient engagement are indeed morally important for research, I ascertain the particular circumstances in which patient engagement is morally important as a means for achieving such ends. Regardless of the moral importance of the ends of patient engagement, there could be something intrinsically valuable within patient engagement itself that motivates its moral importance, so I also turn my attention to this separate question by explicating an argument for the intrinsic value of patient engagement. Ultimately, I hope to show that patient engagement has both instrumental and non-instrumental value: I argue that patient engagement is morally important because it is a strategy for achieving multiple morally important ends and also because it is constitutive of the duty to respect patients as people. At the conclusion, I briefly indicate the implications of this analysis for the practice of patient engagement in research; however, a comprehensive discussion of the bearings of this moral analysis on the conduct of researchers and funders is beyond the scope of this paper.

METHODOLOGICAL APPROACH

Several activities were undertaken to help determine the framing, content, and scope of this moral analysis: a review of the literature on patient engagement; a review of the literature on relevant topics in ethics, bioethics, and moral philosophy; an examination of data from qualitative interviews conducted with researchers and patients on rationales for and effects of patient engagement; and a critical reflection on the rationales for patient engagement that seemed most compelling from a moral perspective.

The review of the extant literature on patient engagement revealed the paucity of discussion on rationales for patient engagement, lending further support for pursuing conceptual scholarship in this area. Two rationales, however, were mentioned in brief by multiple authors: engaging patients to enhance the relevance of the research to patients (Selby, Beal, & Frank, 2012; NIHR 2014b) and engaging patients to promote accountability of researchers and funders (Gradinger et al., 2013; Tritter & McCallum, 2006; Bastian, 1994; Bastian, 1998; Boote et al., 2002). The literature's attention to these rationales suggested their importance, and the lack of arguments to support them indicated that they would be appropriate for more in-depth exploration.

The review of the literature in ethics, bioethics, and moral philosophy aided the identification of relevant ethical concepts needed to construct arguments that were firmly grounded in ethical theory and that constructively advanced concepts in the field of bioethics. The review also informed the framing of this paper. In ethics, value is differentiated by instrumental value (e.g., value as a means for obtaining something else that is held as good) and non-instrumental value (e.g., something that is good for its own sake) (Schroeder, 2012). This distinction was adopted to frame the arguments explicated

in this analysis. Instrumental value refers to patient engagement having value because it is a strategy for achieving a morally important outcome, and non-instrumental value refers to patient engagement having value because it is morally important itself, regardless of its effects.

An examination of data from qualitative interviews I conducted with researchers and with patients engaged in the researchers' projects aided in the selection of rationales explicated in this analysis and also helped to both validate and challenge the thinking and assumptions presented in this paper. In interviews, researchers and patients cited enhancing the relevance of the research to patients as both a main reason for and effect of engagement (Ellis & Kass, forthcoming). Given the prominence of relevance—both in my prior empirical work and in the extant literature—this rationale was selected to explicate in this analysis. While not as commonly mentioned, accountability was also discussed as an effect of patient engagement and was selected as a rationale to explore further because of its mention in both the literature and in interviews and because of its importance in moral philosophy. Last, interview data also revealed that the concept of respect emerged repeatedly from both researchers and patients in discussing the value of patient engagement. The importance of respect in the field of bioethics, critical reflection of this concept as ethically relevant for arguments for patient engagement, and inference to respect in discussions on the value of patient engagement all contributed to the decision to explore arguments invoking respect.

The use of the empirical data to inform the moral arguments advanced in this work allowed for the moral arguments to be tested and challenged as they were being developed and also ensured that the analysis was consistent with findings from

interviews. The empirical data also helped inform the scope of this work. Interview participants discussed engagement in comparative effectiveness research; however, their responses suggested the value of patient engagement more broadly, so this analysis is not limited to comparative effectiveness research and instead considers the value of patient engagement in research more generally.

The methodological approach involved an examination of the literature and interview findings combined with critical reflection to explicate arguments for the instrumental and non-instrumental value of patient engagement in research. The arguments and concepts explored in this conceptual work are not the only arguments that one could use to consider the moral importance of patient engagement; however, they appear to be the most salient, which is why they were selected for this conceptual project aiming to explicate the moral importance of patient engagement in research.

INSTRUMENTAL VALUE OF PATIENT ENGAGEMENT

This section explicates three arguments for the instrumental value of patient engagement in research. Instrumental value refers to patient engagement having value because it is a strategy for achieving a morally important outcome. In each argument explored in this section, patient engagement is held as valuable because it leads to outcomes that are morally important for research. Specifically, I argue that patient engagement is a strategy to enhance the relevance of the research to patients, to hold researchers and funders accountable, and to help researchers learn ways to show respect to study participants, and that each of these outcomes is morally important for research.

Relevance

It is commonly said that patient engagement is a way to understand patients' needs and interests in order to produce research that is more relevant to patients. In interviews with researchers who had engaged patients and also with some of the patients they engaged, there were references to increased relevance as both a reason to engage patients and as a main outcome of engagement.¹ Both researchers and patients described two ways in which patient engagement enhanced the relevance of the research. First, patient engagement was thought to help ensure that the research project as a whole was important to patients:

By including the community, and especially in how we formed the question, we were able to come up with something that was what the community perceived as being an issue for them. – Researcher 105

We *must* be more inclusive of the areas of health that matter most to patients and attend to the outcomes that are of relevance to *them*. You can't possibly do that unless you are more engaging. – Researcher 102

Research has gotten too far off on its own, in its own little silo. Those little guys in locked doors, huddled over the petri dishes... and then they come up with something and it is presented to the real world, the patient world, and patients are going, "Yeah, so what?" So we are trying to build relevancy. – Patient A 102

Second, informants specifically described how patient review of study instruments helped to ensure that the questions asked in study instruments were relevant to patients:

This tool has been... validated extensively. We say that all the time. But that person might say, "But you know what you're missing? You never asked this." – Researcher 109

Sometimes the [survey] questions about how you've been treated medically don't really get at a patient's concerns at all. In fact, they completely miss them. And so lots of times we've been able to say, "Well, that question doesn't work; a

¹ Interview references and quotations throughout this paper are from qualitative interviews with 19 researchers engaging patients in their research and with 33 patients engaged in the same researchers' studies. Researchers and patients were asked about their experiences with engagement including reasons for engaging patients and the effects of engagement on the research process. Quotations are identified by the respondent type ("Researcher" or "Patient A" or "Patient B") and by a number randomly assigned to each participating study.

better question would be this one.” – Patient A 103

If I hadn't been on the study and I hadn't brought up the point that a lot of the people... don't read well, what would the study [instrument] questions have been like? And are you really capturing the right kind of information if you don't know your population? – Patient A 202

These examples highlight how patient engagement can be a mechanism for patients to provide input on the interpretation of instrument items and the extent to which they are able to capture what patients recognize as important.

The fact that patient engagement can enhance the relevance of the research overall and of research instruments makes patient engagement seemingly desirable, for what researcher would not welcome the opportunity to make her research project more useful and meaningful to the intended end users of her research? While there are practical benefits to enhancing the relevance of research in the ways described above, if patient engagement is to be *morally important* on grounds that it enhances the relevance of research, then relevance must be held as morally important for the conduct of research. The question explored in this argument is whether relevance is morally important for research.

When patients give input that is said to enhance the relevance of the research question, they are expressing what they take to be *important* or *valuable* to them and other patients. Thus, patients' views of the relevance of a research study may approximate the social value of that study, at least in the eyes of patients. This is consistent with prominent accounts of value in the research ethics literature, which reflect that assessments of relevance can contribute to or even define the social value of a research study. Freedman (1987), for example, defines value as the importance or usefulness of the research hypothesis. Other accounts of value include the relevance of

the research question to the patient population and the public's perception of the condition as factors that can enhance value (Casarett, Karlawish, & Moreno, 2002; Grady, 2002).

Three qualifications are pertinent to this discussion. First, an assessment of a study's relevance, as a social construction, is a function of the assessor, so multiple stakeholders' views on the relevance of a research study are potentially important to determining the overall social value of that study. What patients view as a relevant research question may differ from what researchers and policymakers consider relevant or even from what patients' clinicians might have considered relevant; therefore, a complete assessment of relevance requires more than just *patient* input. An assessment of relevance that takes multiple stakeholders' views into account—namely, a global assessment of relevance—is ideally what should contribute to determinations of social value.

Second, other factors in addition to stakeholders' views of the relevance of the research contribute to determinations of the social value of the research. For example, Casarett and colleagues (2002) include the generalizability of findings, the existence of mechanisms for translation of results, and the availability of the intervention (if found effective) as features that contribute to the social value of a research study, and Freedman (1987) includes the scientific validity of a study as a formal requirement of value.

Third, social value is a matter of degree, rather than a binary distinction. The question is often not whether or not a study *has* social value at all, but rather *how much* social value a study has. Further, it is not clear what the threshold level of social value is or the precise extent to which stakeholders must find a study relevant in order for

relevance to be held as enhancing social value. This suggests that a study that is relevant to some stakeholders but of little relevance to others can still be socially valuable; however, its social value would be increased if all stakeholders were to find the study relevant.

Social value is widely regarded as ethically required for all clinical research (Emanuel, Wendler, & Grady, 2000).² Insofar as patients' perspectives on relevance contribute to a global assessment of relevance, which helps to determine the social value of a research project, patients' view of relevance is morally important. Because studies that are of little relevance to some stakeholder groups but highly relevant to other groups can still be considered socially valuable and because other factors may influence assessments of social value, there is no clear guide for determining precisely how much the *patients'* view of the relevance of the research alone matters for the global assessment of relevance and thus contributes to determining the social value. Despite these qualifications, it is still possible to delineate key characteristics of research that, when present, strongly suggest that patients' view of relevance enhances the overall social value of that research. This is to say that there are some kinds of research for which getting patients' perspectives on the relevance of the research, while alone not determinative of the global assessment of relevance or even of social value, is important

² While some in the field of bioethics hold social value as morally important without explicitly referencing any underlying ethical values as justification for its moral importance, Emanuel and colleagues reference two ethical values: the responsible use of scarce resources and the avoidance of exploitation. Wertheimer (2014) has argued that these specific ethical values do not, in fact, provide a robust ethical justification for the social value requirement; however, there are perhaps other ethical justifications one could use to argue that social value is morally important or required, and so it remains widely accepted in the field of bioethics. The social value requirement is also codified in several ethical guidelines and assessed by institutional review boards during review of research plans.

from a moral standpoint. In such circumstances, patients' point of view on the relevance of research carries special significance. These circumstances include studies for which findings are likely to have immediate implications for patients' health and wellbeing and studies in which patients' values or preferences are at stake.

As mentioned earlier, *social value* is a multifaceted concept, and as such it is generally understood to include both studies that contribute to scientific knowledge but have no immediate implications for health and wellbeing and also studies that do have more direct implications for prevention, treatment, or diagnosis. The social value of studies with direct ramifications on patients' health should be assessed by their relevance to patients because patients have a considerable stake in the outcome of the research, perhaps more so than other stakeholder groups. Further upstream research such as early phase clinical research does not have direct implications for patients' health, and so patients' assessment of relevance is less important. Later phase clinical research (e.g., comparative effectiveness research and pragmatic clinical trials) tend to have more direct implications for patients, so patients' assessment of relevance is important for the social value of these kinds of studies. Patients' assessment of relevance is also important when the research involves patients' values or preferences. For example, patient decision aids take for granted that what is important to patients is reflected in the aids, so patients' assessment of the relevance of the overall research question of studies developing or testing decision aids is important for determining the social value. While these distinctions regarding the circumstances in which patients' assessment of relevance enhances the social value of that research are admittedly a bit blurry, they serve as a heuristic that can help identify the kinds of research in which patient input is morally

important on grounds that it enhances the relevance of the research from the patients' perspective.

In considering the moral importance of the other sense of relevance—that is, relevance of study instrument items—the same kind of argument applies. Relevance in this sense is morally important not only as a means to enhance social value, but also as a means to enhance the *scientific validity* or *quality* of the research, including the face validity and content validity of study instruments. This is to say that by making instrument items more reflective of what patients view as important, patient input can help the study to more accurately capture what is important to patients, thereby increasing both social value and validity. The scientific validity of a study has been described as a component of social value (Freedman, 1987), and is also held as a separate ethical requirement of all clinical research (Emanuel et al., 2000). Patient input on study instruments, accordingly, is morally important so far as it improves the validity of study findings.

Like social value, however, the question of scientific validity is often a matter of degree, without a clear threshold. Studies without patient input could certainly still be scientifically valid, even if they could be made *more* scientifically valid by way of patient engagement. For example, a patient with a chronic disease reported in an interview that while the study instrument included questions on many aspects of her disease that were important to patients like level of pain and functioning, there were no questions on *sleep* and *fatigue*, which patients felt were important indicators of how well their disease was managed. If researchers had not included questions on sleep and fatigue in the study instrument, the results of the study with respect to pain and functioning would not have

been invalid, but they would not have told the whole story. In other words, the research may produce incomplete conclusions by the omission of additional areas of relevance to patients. A further complication is that it is usually not possible to know, prospectively, the extent to which patient input will impact the relevance of study instruments. Study instruments developed without patient input may include all the domains that patients consider important.

While it is not possible to know whether patient input will have marginal, moderate, or substantial impacts on the validity of study instruments until after patient input is sought, the fact that patient input can enhance validity suggests it is morally important, at least in some circumstances. Studies in which information from patients is collected through interviews/focus groups and surveys are good candidates for obtaining patient input as a means to potentially enhance the relevance of study instruments because such studies rely on information patients provide themselves. This includes patient-reported outcomes, self-assessments, and patient preferences or opinions collected as data.

This argument shows that relevance is important to the social value and also the scientific validity of research, both of which are widely accepted as ethical requirements of all clinical research. Therefore, patient engagement, as a mechanism to enhance relevance, is also morally important, at least in circumstances in which patients' perspectives on relevance are important for social value and/or scientific validity. Specifically, research that has direct implications for patients' health and research in which patients' values and preferences are involved may improve their relevance and, by extension, their social value, by seeking patient input. This is not to say that patients are

the only ones who can assess the relevance of the research or that patients' view of relevance supersedes what other stakeholders view as relevant. While patients' assessment of the overall relevance of a study is just one component of the study's social value and their view of relevance may differ from that of other stakeholders, to exclude patients from discussions about the relevance of research would be to decide what is socially valuable research without input from a key stakeholder whose view of relevance necessarily factors into the global assessment of a study's relevance and subsequently, its social value.

For research that collects patient-reported data, engaging patients may enhance the relevance of study instruments by prompting minor or substantial improvements to the scientific validity and overall value of the study. This is not to suggest that studies without patient input are invalid or that patient input is only important to ensure that study outcomes are relevant. Rather, it is to suggest that in addition to enhancing the relevance and social value of the research as a whole, getting patient input on study instruments holds the possibility of enhancing the face validity and content validity of study instruments, which may improve the overall scientific validity of the study.

Accountability

Some authors have argued that patient engagement in research serves as a means to enhance accountability. A recent narrative review identified accountability and the related concept of transparency as underpinning values of public involvement in research (Gradinger et al., 2013), and accountability is cited as one of the main reasons for patient involvement in research in the United Kingdom (Bastian, 1994; Bastian, 1998; Boote et

al., 2002; Tritter & McCallum, 2006). In colloquial use, “accountability” refers to the expectation that individuals are answerable or responsible for decisions they make, and “transparency” refers to making information visible or accessible. Both transparency and accountability were mentioned as benefits of patient engagement in interviews with researchers and patients:

The patients are there to discuss and impact the goals that are set in the grant, the promises that are made, the expectations that are created in it. – Patient B 203

Last week there was that article about whether some of the data had been falsified... And I do think having to present what you're doing periodically to stakeholders [including patients] who are smart people may in some ways keep some of that from happening. – Researcher 204

The scientist that is behind all of it has to be willing to incorporate the information that they're getting from those stakeholders... I think it's very simple... You just say- you actually do what you say you're going to do. – Researcher 209

These statements reflect how both researchers and patients viewed patient involvement in the research as a way to compel researchers to be open and honest and to remain committed to doing what they promise to do in the course of the research.

Before exploring whether accountability is a morally important end of patient engagement, it is necessary to clarify the more technical, non-colloquial meaning of “accountability” in the context of research. A large body of literature in political philosophy explores accountability as a key dimension of formal representation in democratic processes (Dovi, 2014). Accountability in this *political* sense is not germane to patient engagement in research, for this notion of accountability refers to formal ways in which citizens hold political institutions and officials to account, such as the electoral process. The research enterprise is not a political institution and involves no formal democratic processes; thus, accountability in a *political sense* is not the kind of accountability that patient engagement is thought to enhance.

Accountability in a *social* sense is more suitable for considering patient engagement as a way to enhance the accountability of researchers or funders. Whereas political accountability refers to formal mechanisms that exist in the political environment, social accountability refers to “alternative ways for social actors (citizens, civil associations and the media) to direct demands” (Jelmin, 2012, p. 7). This includes a wide range of activities such as public demonstrations, discourse, and participation in decision-making (The World Bank, 2006). On this view, patient engagement can be understood as an informal way in which patients hold researchers or funders to account.

This analysis will focus on *social accountability* because this conception most closely approximates what is meant in discussions about patient engagement as a means to hold researchers accountable. Accountability in this sense requires three specific commitments on the part of researchers. First, accountability requires researchers to practice transparency regarding the decisions they make. Patients, at least, are informed about the decisions being made or, at most, play a key role in making such decisions. Second, accountability requires researchers to *explain* the decisions they make in the context of their research. This means that the act of engaging patients provides an opportunity for researchers to justify their reasoning to patients, and may, in some cases, prompt changes to their reasoning or decisions in response to patients’ input. Last, accountability requires researchers to make good on their promises—to do what they said they would do.

Is accountability, thus understood, morally important for clinical research? This is to ask: is it morally important for researchers to be transparent, to explain their reasoning, and to be held to their word? There are two compelling reasons for endorsing

accountability as morally important. First, accountability promotes the utility of the research enterprise and fairness in the distribution of research benefits. Second, researchers may have moral obligations to patients and to the general public pertaining to the way they conduct research and use public resources. Each of these reasons is further explicated below.

The research enterprise functions more effectively when researchers are open, answerable, and committed to their decisions. When researchers are accountable, patients may be more likely to have confidence in the research system, accept research findings, and support future research through funding or participation, all of which are essential for sustaining the research enterprise. In fact, openness and “proactive and expansive approaches to accountability” that extend beyond regulatory requirements have been identified as key strategies for promoting public trust in biomedical research (Yarborough, Fryer-Edwards, Geller, & Sharp, 2009, p. 474). Further, there is some evidence of increased mistrust in the research system among racial and ethnic minority populations compared to the white population (Braunstein, Sherber, Schulman, Ding, & Powe, 2008; Kennedy, Mathis, & Woods, 2007; Ulrich et al., 2013). Enhanced public trust in biomedical research may help to address the underrepresentation of minority populations in research, which could help to generate more generalizable research findings and promote fairness in the distribution of research benefits. Researchers also demonstrate respect to patient populations when they take steps to establish and maintain trustworthiness. Given that accountability fosters public trust essential for the utility and fairness of the research enterprise, mechanisms for accountability are generally morally important. Such mechanisms are especially important in circumstances where the patient

population lacks trust, perhaps as a result of past wrongdoing, such as in the case of research with some minority populations.

Second, there are circumstances in which researchers have moral obligations to account to study participants and to the general public during the conduct of their research. These circumstances include when researchers make promises to study participants and thus become obligated to keep their promises and also when researchers use public resources and thus have obligations to the general public to conduct their research as intended and to use resources responsibly.

While conducting research, researchers inevitably make promises to study participants; they are morally obligated to keep such promises. Researchers set study participants' expectations regarding how the research will be conducted and regarding what will happen to study participants over the course of the research. Perhaps more importantly, researchers also make promises regarding what will happen when the research is over including how research results will be shared with study participants and other stakeholders and how results will be used. Such expectations are often set by information provided during the recruitment and informed consent processes, and patients may even decide to participate in research because of what researchers promise during such discussions. These promises can range from minor (such as promising to provide reimbursement for parking) to more serious (such as stating that the research results will be used to improve patient care). When researchers articulate expectations, whether minor or significant in nature, they create moral obligations to uphold them.

The generation of such moral obligations is not unique to the relationship between researchers and study participants; rather, the moral obligation to keep a promise is a

feature of any relationship in which one person explicitly makes a promise to another. In certain professional roles, however, keeping one's word may arguably be even more important because it helps preserve trust (e.g., religious, legal, and medical professionals). Researchers may fall into this category of professional roles because study participants' trust in them is preserved when they uphold their commitments over the course of the research. Whether or not one accepts researchers as being in a professional role that heightens expectations to keep promises, promise keeping is a general moral obligation of all promise makers, and so researchers are morally obligated to keep their promises by adhering to research plans they convey to people participating in their research.

The final circumstance in which researchers and also funders can be said to have moral obligations to account is when public resources support researchers' work. When funders award researchers public funds to conduct their proposed research, researchers are morally obligated to use the funds to conduct the research in accordance with what they proposed. Whenever public funds are used, the public bears the opportunity cost of not pursuing alternative uses of limited public resources. Researchers and funders must be accountable to the general public as a means to ensure that public resources are being spent responsibly and as intended. This obligation falls on government funding organizations and other organizations that are resourced through public dollars because they have a role in deciding how public dollars are spent. The moral obligation to account may be discharged in myriad ways from public deliberation at the level of the funding agency to patient engagement in individual research projects. PCORI and NIHR, as publicly supported funding organizations, can be said to be honoring moral obligations

to account for how public resources are spent through their requirement for public involvement in the research process.

As an accountability mechanism, patient engagement may not only help researchers to *keep* their promises, it may also provide a venue for researchers to *make* promises. When researchers engage patients during the research process, they provide an opportunity for patients to express their concerns, give feedback, and make ancillary requests or suggestions. Researchers who are receptive to patients' input may make commitments in response to patients' feedback. For example, patient advisors may recommend to researchers that they share research results with influential advocacy organizations and medical associations in order to promote better uptake of research findings into practice, and if researchers agree to do so, they are obligated to follow through on their commitment. This is to say that aside from patient engagement serving as a strategy for researchers to meet *existing* moral obligations, it may generate *new moral obligations* of researchers. Patient engagement may also prompt researchers to commit to doing beneficial things that they otherwise would not have thought to do.

In sum, accountability is morally important for research insofar as it fosters public trust essential for sustaining the research enterprise and also promotes fairness in the distribution of research benefits. Researchers have moral obligations to uphold commitments made to study participants and to conduct research supported by public funds in accordance with what they proposed to do. In such cases, patient engagement can serve as a mechanism to hold researchers accountable, and it may create the opportunity for researchers to make and keep additional promises to patients. The moral

requirement for researchers to be held accountable may be satisfied through a range of mechanisms, one of which is patient engagement.

Respect for Research Participants

There is evidence suggesting that patient engagement can result in research that is conducted in a more respectful manner. Examples reported in interviews illustrate some of the ways in which patients engaged in research informed researchers about specific steps they could take to ensure that they were treating their study participants with respect. Interview participants reported how patient feedback helped researchers recognize unintentional signals of disrespect embedded in their behavior:

There are those that keep their relationship with their typewriter and just forget the patient is even there... I came back to the group and I told them that.
– Patient A 207

I don't think we, as researchers... understand how our actions demonstrate to people that we don't value their opinions... I've learned a lot about the behaviors and the actions that researchers do to show that to patient groups and children inadvertently. – Researcher 101

In addition to teaching researchers about behaviors to avoid so that study participants do not feel disregarded, patient research partners also recommended changes to study protocols to accommodate study participants' needs or preferences. For example, patient advisors for one study insisted that researchers provide transportation for study participants and that remuneration be given in person in cash rather than a check sent through the mail in order to reduce inconvenience for study participants. In some cases, substantial changes to study designs were made in consideration of study participants' comfort:

There was a discussion about... people not being as forthcoming in a group with people that they don't know as opposed to being one-on-one with an investigator

and kind of being able to share more and feeling more comfortable in a one-on-one situation, so we... decided that maybe that was the direction to go.
– Patient B 204

Through engaging patients, researchers were informed of many ways to demonstrate respect and were able to incorporate respectful practices into their research plans and interactions with participants, suggesting that patient engagement can serve as a tool for enhancing respectful treatment of study participants.

Respect for research participants is widely accepted as morally required (Beauchamp & Childress, 2009; National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research, 1978). The questions that need to be addressed for the purpose of this analysis are first, whether the canonical conceptualization of respect can be said to entail obligations on the part of researchers to behave in the ways described above, and second, if such behaviors conform with researchers' obligations of respect, whether patient engagement is morally important for researchers to meet such obligations.

The bioethics principle of respect is traditionally understood as respect for autonomy. Beauchamp and Childress (2009) articulate the principle of respect as acknowledging peoples' "right to hold views, to make choices, and to take actions based on their personal values and beliefs" (p. 103). Obligations on the part of researchers that are entailed by the principle of respect for autonomy include obtaining informed consent and allowing study participants to withdraw (Beauchamp & Childress, 2009; Emanuel et al., 2000). These actions demonstrate respect for autonomy by recognizing and enabling study participants to express their views and act in accordance with them.

One of the examples described above clearly relates to respect for research participants in this autonomy-directed sense. The researcher who learned that researchers

inadvertently “demonstrate to people that we don't value their opinions” was implying the moral importance of recognizing study participants’ beliefs and their right to have them. The other kinds of respectful behaviors described above, however, do not seem to relate to respect for autonomy. Rather, they speak to actions that are respectful to persons in the sense that they demonstrate attention to study participants’ needs, feelings, preferences, or level of comfort. These behaviors seemingly fall outside the traditional purview of duties entailed by the principle of respect for autonomy as articulated by Beauchamp and Childress, yet they appear consistent with our every day usage of the term *respect* (Dickert, 2009). Avoiding offense, for example, is considered important for ethical behavior in every day life and is done out of respect for others. This suggests that these examples of respect may be ethically important, but a different conception of respect, one that more broadly includes what we think of when we speak of respect, is needed to make this claim.

The fact that respect for people’s beliefs is required under the principle of autonomy while respect for people’s comfort and convenience, for example, is not indicates a distinction between respect for autonomy, as defined by Beauchamp and Childress, and respect for persons, the latter being a more expansive conception of respect containing the former. In tracing the historical usage of the concept of respect in the field of bioethics, Lysaught (2004) identifies a shift from an initially broad conception of respect for persons to the much narrower conception of respect for autonomy.³

³ Lysaught describes how in the early 1970s, Paul Ramsey put forth an expansive conception of respect for persons that included acknowledging a person’s beliefs and attending to his or her needs. By 1979, however, when Beauchamp and Childress’ first edition of *Principles of Biomedical Ethics* was published, the principle of respect was defined in the context of autonomy.

While respect for autonomy remains the predominant account of respect in the field of bioethics, some scholars have argued that it is too narrow and have proposed broader conceptions of respect (Beach, Duggan, Cassel, & Geller, 2007; Dickert, 2009; Lebacqz, 2005). Dickert (2009), for one, defines respect as “a combination of appreciating what is valuable or important about a person, recognizing the constraints or demands that such a valuation places on one’s own conduct, and acting in a way that expresses that recognition” (p. 315). Respect for persons thus involves acknowledging what is important to people and demonstrating that acknowledgement through one’s actions. Having knowledge of what is important to people is a requisite for showing respect to them. This broader account of respect demands consideration of autonomy, but it also demands consideration of feelings, emotions, needs, and other subjective experiences that people may value.

While respect for persons is sufficiently broad to include considerations beyond autonomy, it is still difficult to deduce what specific actions it requires. Buss (1999) argues that regular manners such as avoiding “being discourteous, impolite, rude, inconsiderate, offensive, [and] insulting” fall under the scope of what it means to act respectfully, from a moral standpoint (p. 795-6). In the medical care setting, Beach and colleagues (2007) suggest that a health professional may demonstrate respect by “extending common courtesies, expressing concern for others and their wellbeing, taking their feelings and experiences seriously” (p. 694). In the research setting, Dickert (2009) similarly holds that respect for research participants entails “the obligation to attend to issues of comportment” such as using terms that participants can understand when speaking to them, giving them your full attention when speaking, and not talking past

them (p. 325). The specific actions held as required under the principle of respect for persons are comparable to the examples that patients and researchers cited when explaining the effects of patient engagement on research participants. Such actions, therefore, can be said to conform to researchers' obligation to respect research participants.

Given that researchers' obligation to demonstrate respect to research participants can be said to entail behaviors like the ones reported as effects of patient engagement, patient engagement can be said to be morally important insofar as it may help researchers to learn about specific ways to show respect or avoid disrespect to research participants. There are some circumstances in which patient engagement may be particularly useful in helping researchers learn about how to show respect for research participants. These include circumstances in which researchers may be unfamiliar with the study population or may have reason to be particularly concerned about showing respect to them. In such cases, researchers can seek patient input to identify ways that increase the likelihood that their obligations of respect to research participants are able to be met.

Researchers conducting research in communities with which they are unfamiliar may be unaware of what respect in those communities looks like because they lack sufficient knowledge about the study population's needs, experiences, and values. Given that patient engagement may help researchers to learn about what is important to the study population and how to show respect to them, patient engagement can serve as a morally important strategy to help researchers meet obligations of respect to people from these communities. For example, a researcher working with study participants from another culture may learn about behaviors that are perceived as respectful in that culture

and can then adopt such behaviors to show respect to study participants. In this way, patient input may increase researchers' cultural competency to work with the study population (Minkler, 2005). In discussing how community engagement contributes to the ethical conduct of global health research, King, Kolopack, Merritt, and Lavery (2014) make a similar point, noting that community input serves as a way for researchers to learn about research practices that may be offensive and about ways to minimize offense. Patient engagement can help researchers learn about respectful behaviors to adopt as well as disrespectful behaviors to avoid.

Patient engagement is also a useful strategy when researchers have reason to be particularly concerned about respectful treatment of study participants. Research involving communities that have been treated disrespectfully in prior interactions with the healthcare or research system—including populations whose values, needs, or experiences have not been given due consideration in the past—warrant researchers being especially attuned to showing respect. These populations have reason to be suspicious of researchers and the healthcare system more generally, and because of their history of mistreatment, they may be predisposed to feeling disrespected. Research suggests that minority populations have more distrust in the research system than whites (Braunstein et al., 2008; Kennedy et al., 2007; Ulrich et al., 2013), which may be due, at least in part, to historic mistreatment. In fact, a recent qualitative study involving focus groups with African Americans found that lack of trust in the health care system among African Americans is rooted in the legacy of the Tuskegee Syphilis Study, sustained through generations, and exacerbated by disrespectful treatment in the present (Scharff et al., 2010). For populations that have experienced wrongdoing in the past, researchers need to

recognize the historical context that has bred suspicion and mistrust and subsequently do more to demonstrate respect and build trust with such populations. Engaging these populations is a way to reduce mistrust and show respect.

This is consistent with the position of the US Presidential Commission for the Study of Bioethical Issues, which, after reviewing syphilis studies that the United States Public Health Service conducted in Guatemala in the 1940s, recommended that researchers demonstrate respect for study participants and their communities and recognize cultural practices through engaging the population in the research process (Tanne, 2011). Similarly, engagement has also been recommended to help address concerns with regard to genetic research with certain minority populations including American Indians, African Americans, and Latinos (Quinn, 2004; University of Washington Center for Genomics and Healthcare Equity, 2012). Even if researchers themselves are members of these populations, their seeking patient input can not only help them to become more sensitized to concerns that members of the population have but can also show them as trustworthy to skeptics in their communities.

Demonstrating respect is always morally important in research and requires researchers to behave in ways that show respect and to avoid behaviors that are disrespectful. Populations with which researchers lack familiarity or that have experienced wrongdoing from past research may be at greater risk of not feeling completely respected or of feeling disrespected in research, either because researchers are unaware of what respect means in that population or because the population is recovering from prior mistreatment. In these cases, researchers should have heightened attention to ensuring that their obligations of respect are met. Patient engagement, as a means to help

researchers learn about ways to show respect to study participants and build trust, may increase the likelihood that researchers fulfill their moral obligations of respect to study participants.

NON-INSTRUMENTAL VALUE OF PATIENT ENGAGEMENT

Up to this point, this moral analysis has explicated arguments that hold patient engagement as morally important on grounds that its *ends* are morally important. I will now shift my focus to explicating the argument that patient engagement is morally important independent of these ends. Recall that non-instrumental value refers to patient engagement having value because it is morally important itself, regardless of its effects. The question taken up here is whether there is something *non-instrumentally* valuable, some intrinsic property of patient engagement *itself* that is morally important for research and thus provides justification for holding patient engagement to be morally important. Specifically, I argue that certain patient engagement activities are constitutive of the duty to respect the moral worth of patients as people.

Patient engagement consists of researchers including patients in the research process, listening to them, and involving them to varying degrees in research decision-making. Listening to patients is considered a distinctive feature of patient engagement. When patients and researchers described the value of patient engagement, they often referred to the idea of listening to patients as intrinsically valuable:

Regardless of the outcome of the study, I think they feel respected and feel like we care about what they have to say. Even if the study is a total failure otherwise.
– Researcher 106

It's this image of researchers kind of—no offense taken, I hope—in an ivory tower or in a lab, not really listening to the needs of the people who they think

they're creating models to help, and I think when you have an opportunity to chat with people... I think it's incredibly valuable to our community as a whole.
– Patient A 209

To know that somebody is listening out there and they're trying to change things. I think that's very, very encouraging. – Patient A 109

As these quotations suggest, researchers' very act of listening to patients, whether or not it has any demonstrable effects on the research study, is perceived as valuable and is linked to patients' being respected. If listening to patients is constitutive of the duty of respect, then patient engagement can be considered intrinsically morally valuable. As addressed earlier in this paper, it is widely accepted in the field of bioethics that researchers have a duty to respect research participants (Beauchamp & Childress, 2009; National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research, 1978). For the purpose of this analysis, it is necessary to explore how listening to the patient population relates to respect.

Researchers show respect to patients by seeking them out and listening to them. Researchers do not need to agree with what patients tell them or change the research project in response to patients' views in order for researchers to respect patients in this way. My earlier discussion of the concept of respect showed that engaging patients can help researchers to learn about specific ways to show respect over the course of the research project. The claim I am making now, however, is that engaging patients itself shows respect to patients because the act of listening to patients is one way for researchers to recognize their value. That is, it is not just the result of listening to patients that may show respect to patients, it is also the fact that researchers are seeking patients out and displaying a desire to hear them that demonstrates respect to patients. The notion

that researchers respect patients by seeking their opinions and listening to them is deeply connected to what it means to respect patients as people.

Described as the foundation on which contemporary discussions of respect are often based, Kant's ethical theory provides an account of what it means to respect someone as a person (Dillon, 2014). Kant (1785/1993) held that all people have distinctive moral worth and should be treated in a way that recognizes such worth—namely, by treating people “never simply as a means but always at the same time as an end” (sec. 429). The duty to respect the moral worth of an individual is a general ethical obligation that all people have to others and also to themselves (self-respect). Beach and colleagues (2007) advance a Kantian conception of respect in the context of clinical care, holding respect as the “recognition of the unconditional value of patients as persons” (p. 692). Because this duty entails respecting patients *as persons*, it is not dependent on any particular characteristic of patients. This Kantian conception of respect requires that we each recognize that every person has moral value simply by virtue of his or her personhood.⁴

Given that respect requires that all people acknowledge the moral value of other people, researchers are required to acknowledge the moral value of patients as people too. The act of seeking out patients and listening to them respects patients precisely because it is a way for researchers to recognize the unconditional moral value that patients have as persons. Because the act of listening to patients recognizes patients' moral value, patient engagement has intrinsic moral value.

⁴ This analysis treats the duty to recognize the unconditional value of people as a starting point. Recreating Kant's argument for the moral worth of all humans is beyond the scope of this paper as is discussion of whether some humans do not have moral status (e.g., cognitively impaired individuals, fetuses) and thus are not entitled to respect as moral persons.

This argument holds that listening to an identified *subset* of patients is constitutive of respect to the *larger* and perhaps indeterminate patient population that includes that subset. While patient research partners cannot be said to represent, in a formal sense, the larger patient population from which they come, the fact that some patients are being sought out and heard indicates that researchers recognize that *all* patients from that population have moral worth. By engaging even a few patients in the research process, the researcher is demonstrating to the larger patient population that she values them enough to want to hear what patients from that group have to say. This claim is at least intuitively plausible. Patients who are not engaged in research may still appreciate the fact that researchers are listening to patients like them and gathering some patients' perspectives, and patients who are engaged in research may recognize that not all patient views are likely to be captured by engaging even several patients from their population. Researchers and their patient research partners alike must be mindful that the patients engaged in research are not necessarily representative of the larger patient population of which they are a part; however, lack of representativeness does not undermine patient engagement as constitutive of the demonstration of respect to the general patient population. Researchers concerned about representativeness among patients they are engaging can also take steps to have ample inclusion of patients with a range of predetermined characteristics (e.g., health status, geographic region, socioeconomic indicators) to help ensure that a diversity of perspectives are included (Crocker, 2008).

The act of listening to patients makes patient engagement intrinsically morally valuable as it recognizes the unconditional moral worth that patients have as people deserving of respect. Because listening to patients is morally important, researchers

conducting research with populations that have been ignored or silenced in the past may consider engaging patients as a way to listen to them and show respect to them. From a practical standpoint, listening to patients who have not been shown respect in the past or who feel that they have not been heard is a reasonable way to show that one does, in fact, care about what patients have to say. In fact, empirical research suggests that feeling listened to is a salient feature of feeling respected (Frei & Shaver, 2002; Parse, 2006). When patients feel that researchers are listening to them, they feel valued and cared about. From a moral perspective, patient engagement offers a way for researchers to make explicit that they recognize patients as deserving of respect.

Patient populations that have not been shown respect or heard likely overlap with some of the other populations for which patient engagement is already morally important—namely, populations that have been mistreated in the past and minority populations that lack trust in the research system. Additional populations that fit into this category may include socially marginalized or stigmatized groups and rare disease groups whose small numbers may impede their ability to receive due attention. Researchers can engage patients from populations such as these as a way to listen to patients and demonstrate the respect that is due to all patients as people. There are some well-known cases that show the power of patient engagement in giving voices to previously unheard or silenced groups that subsequently received the respect they deserved. The early years of the HIV/AIDS crisis in the United States, for example, brought unprecedented activism from the socially stigmatized gay community that ultimately resulted in their engagement in HIV/AIDS policy and research and their being treated as people deserving of respect (Epstein, 1995).

In summary, respecting the moral worth of all people is a moral obligation that that can be discharged in different ways. In clinical research, one such strategy for recognizing the unique moral value of patients as people is to seek them out and listen to them. This feature of patient engagement makes it intrinsically morally valuable.

CONCLUSION

This analysis described the role of patient engagement in enhancing the relevance of research, the accountability of researchers and funders, and the respectful treatment of research participants. Then it argued that each of these effects of patient engagement is morally important for research, thus making patient engagement a morally important strategy for achieving each of these ends.

Relevance is important to the social value and the scientific validity of research, both of which are moral requirements of research. Patient engagement, as a strategy to enhance relevance, is thus morally important in some kinds of research. In particular, patient engagement is morally important as a way to enhance relevance when the research has immediate health implications, involves patients' values or preferences, or collects patient-reported data.

There are some circumstances in which researchers have moral obligations to account for their actions. Specifically, researchers must be held accountable for the promises they make to study participants and for how they spend public funds in the conduct of their research. In these circumstances, patient engagement can serve as a mechanism for researchers or funders to meet moral obligations of accountability and as a venue for additional promises to be made. While patient engagement is not morally

required in these circumstances, some form of accountability is, and patient engagement is one such strategy available for researchers or funders. Because accountability can also enhance public trust, promote fairness, show respect to patients, and help sustain the research enterprise, patient engagement, as a mechanism for accountability, is morally important even when no moral obligations of accountability are present.

Researchers are morally required to treat study participants with respect. Patient engagement may increase the likelihood that researchers are meeting their moral obligations to treat research participants respectfully, obligations that are heightened when researchers lack familiarity with the study population or are working with historically disrespected populations who have reason to be suspicious of research. This is not to say that patient engagement in these circumstances is the only way to meet obligations of respect or that patient engagement is morally required, but it is to say that researchers must meet obligations of respect, one way or another, and patient engagement is one such way to help researchers meet their obligations, especially in these circumstances.

In exploring whether patient engagement has non-instrumental value, this analysis found that patient engagement is constitutive of researchers' moral obligation to recognize the patient population as persons with moral worth and thus has non-instrumental value. When researchers listen to patients, they are showing that they recognize the unique moral value of patients as people. Researchers may discharge their moral obligation to respect the moral worth of patients by engaging patients, and this strategy may be especially important for populations who have not been respected or heard in the past. This is not to imply that patient engagement is the only way to

recognize the moral value of patients as people. Researchers may build strong personal relationships with patients and demonstrate respect for them as people through their personal interactions, or researchers with reputations for disseminating findings back to study participants can be said to be treating them as ends rather than merely as means for conducting their research, thus demonstrating respect for patients as people. Thus, while not necessary for fulfilling researchers' obligations of respect, patient engagement is an additional way for researchers to respect the patient population as people with moral worth.

This analysis revealed that patient engagement is helpful for enhancing relevance, accountability, and respectful treatment of research participants. I argue that each of these ends is morally important for research, as is the act of seeking patients out and listening to them, an intrinsic feature of patient engagement. Patient engagement, therefore, is morally important both as a strategy for achieving certain morally important ends and as a behavior that is constitutive of the duty to respect patients as people. Patient engagement could only be held as morally required if it were the only way to achieve these morally important results. Because there may be other ways to enhance relevance, hold researchers and funders accountable, treat research participants respectfully, and acknowledge the unique moral value of patients, it cannot be said that patient engagement is morally required. However, given that this analysis revealed the potential utility of patient engagement in satisfying multiple morally important results, patient engagement is a highly efficient strategy and a worthwhile endeavor, from a moral perspective.

In addition to explicating the moral importance of patient engagement, this analysis distilled the circumstances in which there are strong moral reasons for engaging patients and provided examples of the kinds of research in which these circumstances may exist. Accordingly, this analysis has implications for the practice of patient engagement. The strength of the moral reasons for engaging patients in research is dependent on a number of particular facts about any given research project that subsequently determine the extent to which the results of patient engagement are morally relevant. These include considerations regarding the extent to which findings will have immediate implications for the health of patients, the kind of data collected from the research, whether public resources are being used, whether researchers have made promises to study participants, the level of trust the patient population has in the research system, researchers' familiarity with the study population, and the extent to which the patient population has had an opportunity to be heard or has been respected in the past (Table 3.1). Depending on these considerations, there may be strong or weak claims for the moral importance of engaging patients in research as a means for achieving morally important ends and respecting patients as people. Further, whether alternative strategies are in place to achieve these morally important goals will also affect the strength of the argument for engaging patients in a particular research project. By elucidating the moral underpinnings of patient engagement in research, this analysis suggests the kinds of research in which patient engagement is morally important. Given the conceptual developments provided in this paper, researchers, funders, and institutional review boards may consider policies endorsing patient engagement in research meeting certain conditions in accordance with the moral rationales provided here.

TABLES

Table 3.1: Overview of the Moral Reasons for PE (Patient Engagement) in Research

Reason for PE	Moral importance of reason for PE	Circumstances in which PE is a morally important strategy for acting in accordance with said reason	Examples of research in which circumstances may exist
Relevance of research study	Patients' view of relevance contributes to social value, and social value is <i>morally required</i> for all clinical research.	PE is morally important for studies in which patients' view of relevance is likely to enhance the social value of that research.	Studies with immediate implications for health Studies in which patients' values or preferences are involved
Relevance of instrument items	Relevance of items may enhance scientific validity and contribute to value, and both are <i>morally required</i> for all clinical research.	PE is morally important for research collecting data from the patients' perspective.	Studies using surveys or interviews to gather patient perspectives Studies using patient-reported outcomes
Accountability	Accountability promotes utility and fairness of the research enterprise, making it <i>morally important</i> for clinical research. In certain circumstances, researchers are morally obligated to be held accountable.	PE is morally important when public trust and sustainability of the research is of particular concern. PE can serve as a mechanism to fulfill the moral obligation of accountability when it exists.	Studies with populations that lack trust (that have been mistreated) Studies in which researchers make promises to study participants Studies funded by the public
Respectful interactions	Respect for research participants is <i>morally required</i> for all clinical research.	PE is morally important insofar as it is one strategy for helping researchers learn specific ways to respect study participants.	Studies with populations that have been mistreated Studies in which the researcher is unfamiliar with the patient population
Intrinsic value	PE itself is <i>intrinsically morally valuable</i> because it recognizes the unconditional value patients have as people.	PE is morally important as one strategy to listen to patients, thereby respecting the moral value of patients.	Studies with populations who have not had the opportunity to be heard or who have been silenced in the past

This table provides an overview of the moral importance of each reason for patient engagement and the circumstances in which engaging patients is morally important for acting in accordance with each reason. The far right column serves as a practical guide providing examples of the kinds of research in which the circumstances that make patient engagement morally important are likely to be present.

REFERENCES

- Bastian, H. (1994). The power of sharing knowledge: Consumer participation in the Cochrane Collaboration. *Cochrane Collaboration*. Retrieved from http://consumers.cochrane.org/sites/consumers.cochrane.org/files/BastianPowerofSharingKnowledge_1994.pdf
- Bastian, H. (1998). Speaking up for ourselves: The evolution of consumer advocacy in health care. *International Journal of Technology Assessment in Health Care*, 14(01), 3-23. doi:<http://dx.doi.org/10.1017/S0266462300010485>
- Beach, M. C., Duggan, P. S., Cassel, C. K., & Geller, G. (2007). What does 'respect' mean? Exploring the moral obligation of health professionals to respect patients. *Journal of General Internal Medicine*, 22(5), 692-695. doi:10.1007/s11606-006-0054-7
- Beauchamp, T. L., & Childress, J. F. (2009). *Principles of biomedical ethics* (6th ed.). New York: Oxford University Press.
- Boote, J., Telford, R., & Cooper, C. (2002). Consumer involvement in health research: A review and research agenda. *Health Policy (Amsterdam, Netherlands)*, 61(2), 213-236. doi:S0168851001002147
- Braunstein, J. B., Sherber, N. S., Schulman, S. P., Ding, E. L., & Powe, N. R. (2008). Race, medical researcher distrust, perceived harm, and willingness to participate in cardiovascular prevention trials. *Medicine*, 87(1), 1-9. doi:10.1097/MD.0b013e3181625d78
- Brett, J., Staniszewska, S., Mockford, C., Herron-Marx, S., Hughes, J., Tysall, C., & Suleman, R. (2014). Mapping the impact of patient and public involvement on health and social care research: A systematic review. *Health Expectations: An International Journal of Public Participation in Health Care and Health Policy*, 17(5), 637-650. doi:10.1111/j.1369-7625.2012.00795.x
- Buss, S. (1999). Appearing respectful: The moral significance of manners. *Ethics*, 109(4), 795-826. doi:10.1086/233946
- Caron-Flinterman, J. F., Broerse, J. E., & Bunders, J. F. (2005). The experiential knowledge of patients: A new resource for biomedical research? *Social Science & Medicine*, 60(11), 2575-2584. doi:S0277-9536(04)00570-2
- Casarett, D. J., Karlawish, J. H., & Moreno, J. D. (2002). A taxonomy of value in clinical research. *IRB*, 24(6), 1-6. Retrieved from <http://www.thehastingscenter.org/Publications/IRB/>

- Charles, C., & DeMaio, S. (1993). Lay participation in health care decision making: A conceptual framework. *Journal of Health Politics, Policy and Law*, 18(4), 881-904. Retrieved from <http://jhppl.dukejournals.org>
- Concannon, T. W., Fuster, M., Saunders, T., Patel, K., Wong, J. B., Leslie, L. K., & Lau, J. (2014). A systematic review of stakeholder engagement in comparative effectiveness and patient-centered outcomes research. *Journal of General Internal Medicine*. doi:10.1007/s11606-014-2878-x
- Crocker, D. A. (2008). *Ethics of global development: Agency, capability, and deliberative democracy*. Cambridge: Cambridge University Press.
- Dickert, N. W. (2009). Re-examining respect for human research participants. *Kennedy Institute of Ethics Journal*, 19(4), 311-338. doi:10.1353/ken.0.0295
- Dillon, R. (2014). Respect. In *The Stanford Encyclopedia of Philosophy* (Spring 2014 Edition). E. N. Zalta (Ed.). Retrieved from <http://plato.stanford.edu/entries/respect/>
- Domecq, J. P., Prutsky, G., Elraiyah, T., Wang, Z., Nabhan, M., Shippee, N., . . . Murad, M. H. (2014). Patient engagement in research: A systematic review. *BMC Health Services Research*, 14, 89-6963-14-89. doi:10.1186/1472-6963-14-89
- Dovi, S. (2014). Political representation. In *The Stanford Encyclopedia of Philosophy* (Spring 2014 Edition). E. N. Zalta (Ed.). Retrieved from <http://plato.stanford.edu/entries/political-representation/>
- Ellis, L., & Kass, N. (forthcoming). "Characterizing patient engagement in PCORI-funded research to enhance understanding of its ethical and practical value: A qualitative study of principal investigators and patients." Dissertation chapter. Johns Hopkins University, forthcoming.
- Emanuel, E. J., Wendler, D., & Grady, C. (2000). What makes clinical research ethical? *Journal of the American Medical Association*, 283(20), 2701-2711. doi:jsc90374
- Epstein, S. (1995). The construction of lay expertise: AIDS activism and the forging of credibility in the reform of clinical trials. *Science, Technology & Human Values*, 20(4), 408-437. doi:10.1177/016224399502000402
- Federal Coordinating Council for Comparative Effectiveness Research. (2009). *Report to the President and the Congress*. Washington, D.C.: Department of Health and Human Services.
- Freedman, B. (1987). Scientific value and validity as ethical requirements for research: A proposed explication. *IRB*, 9(6), 7-10. Retrieved from <http://www.thehastingscenter.org/Publications/IRB/>

- Frei, J. R., & Shaver, P. R. (2002). Respect in close relationships: Prototype definition, self-report assessment, and initial correlates. *Personal Relationships*, 9(2), 121-139. doi:10.1111/1475-6811.00008
- Gradinger, F., Britten, N., Wyatt, K., Froggatt, K., Gibson, A., Jacoby, A., . . . Popay, J. (2013). Values associated with public involvement in health and social care research: A narrative review. *Health Expectations: An International Journal of Public Participation in Health Care and Health Policy*. doi:10.1111/hex.12158
- Grady, C. (2002). Thinking further about value: Commentary on "A taxonomy of value in clinical research". *IRB*, 24(6), 7-8. Retrieved from <http://www.thehastingscenter.org/Publications/IRB/>
- Hanley, B., Bradburn, J., Barnes, M., Evans, C., Goodare, H., Kelson, M., . . . Wallcraft, J. (2003). *Involving the public in NHS, public health and social care research: Briefing notes for researchers* (2nd ed.). Eastleigh: INVOLVE Support Unit. Retrieved from [http://www.twocanassociates.co.uk/perch/resources/files/Briefing Note Final_dat\(1\).pdf](http://www.twocanassociates.co.uk/perch/resources/files/Briefing%20Note%20Final_dat(1).pdf)
- Jelmin, K. (2012). *Democratic accountability in service delivery: A synthesis of case studies*. International Institute for Democracy and Electoral Assistance. Retrieved from <http://www.idea.int/resources/analysis/upload/DEMOCRATIC-ACCOUNTABILITY-IN-SERVICE-DELIVERY.pdf>
- Kant, I. (1993). *Grounding for the metaphysics of morals: with, On a supposed right to lie because of philanthropic concerns* (3rd ed.) (J. W. Ellington, Trans.). Indianapolis: Hackett Publishing (Original work published 1785).
- Kennedy, B. R., Mathis, C. C., & Woods, A. K. (2007). African Americans and their distrust of the health care system: Healthcare for diverse populations. *Journal of Cultural Diversity*, 14(2), 56-60. Retrieved from <http://tuckerpub.com/jcd.htm>
- King, K. F., Kolopack, P., Merritt, M. W., & Lavery, J. V. (2014). Community engagement and the human infrastructure of global health research. *BMC Medical Ethics*, 15(1), 84. doi:10.1186/1472-6939-15-84
- Lebacqz, K. (2005). We sure are older but are we wiser? In J. F. Childress, E. M. Meslin, & H. Shapiro (Eds.), *Belmont revisited: Ethical principles for research with human subjects* (pp. 99-110). Washington, D.C.: Georgetown University Press.
- Lysaught, M. T. (2004). Respect: Or, how respect for persons became respect for autonomy. *The Journal of Medicine and Philosophy*, 29(6), 665-680. doi:JWWXCHM7JHDPTNRB

- Minkler, M. (2005). Community-based research partnerships: Challenges and opportunities. *Journal of Urban Health*, 82(2 Suppl 2):ii3-12. doi:10.1093/jurban/jti034
- National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research. (1978). *The belmont report: Ethical principles and guidelines for the protection of human subjects of research* (Publication OS 78-0012). Washington, D.C.: Department of Health, Education, and Welfare.
- National Institute for Health Research (NIHR). (2014a, July 15). Public involvement in your research. Retrieved from <http://www.nihr.ac.uk/funding/public-involvement-in-your-research.htm>
- National Institute for Health Research. (2014b). Get involved: Shape research. Retrieved from <http://www.nihr.ac.uk/get-involved/shape-research.htm>
- Parse, R. R. (2006). Feeling respected: A parse method study. *Nursing Science Quarterly*, 19(1), 51-56. doi:19/1/51
- Patient-Centered Outcomes Research Institute (PCORI). (2014, October 6). About us. Retrieved from <http://www.pcori.org/about-us>
- Patient Protection and Affordable Care Act of 2010, Pub. Law No. 111-148, 124 Stat 727, §6301.
- Quinn, S. C. (2004). Ethics in public health research: Protecting human subjects: The role of community advisory boards. *American Journal of Public Health*, 94(6), 918-922. doi:94/6/918
- Scharff, D. P., Mathews, K. J., Jackson, P., Hoffsuemmer, J., Martin, E., & Edwards, D. (2010). More than Tuskegee: Understanding mistrust about research participation. *Journal of Health Care for the Poor and Underserved*, 21(3), 879-897. doi:10.1353/hpu.0.0323
- Schroeder, M. Value theory. In *The Stanford encyclopedia of philosophy* (Summer 2012 Edition). E. N. Zalta (Ed.). Retrieved from <http://plato.stanford.edu/entries/value-theory/>
- Selby, J. V., Beal, A. C., & Frank, L. (2012). The Patient-Centered Outcomes Research Institute (PCORI) national priorities for research and initial research agenda. *Journal of the American Medical Association*, 307(15), 1583-1584. doi:10.1001/jama.2012.500
- Shippee, N. D., Domecq Garces, J. P., Prutsky Lopez, G. J., Wang, Z., Elraiyah, T. A., Nabhan, M., . . . Murad, M. H. (2013). Patient and service user engagement in research: A systematic review and synthesized framework. *Health Expectations: An*

- International Journal of Public Participation in Health Care and Health Policy*. doi:10.1111/hex.12090
- Sox, H. C., & Greenfield, S. (2009). Comparative effectiveness research: A report from the Institute of Medicine. *Annals of Internal Medicine*, 151(3), 203-205. doi:0000605-200908040-00125
- Staley, K. (2009). *Exploring impact: Public involvement in NHS, public health and social care research*. Eastleigh: INVOLVE. Retrieved from http://www.invo.org.uk/wp-content/uploads/2011/11/Involve_Exploring_Impactfinal28.10.09.pdf
- Tanne, J. H. (2011). US commission recommends increased protection for people in research after reviewing 1940s syphilis study. *BMJ (Clinical Research Ed.)*, 343, d5577. doi:10.1136/bmj.d5577
- The World Bank. (2006). Social accountability: What does it mean for the World Bank? *Social accountability: Strengthening the demand side of governance and service delivery*. Retrieved from http://www.worldbank.org/socialaccountability_sourcebook/PrintVersions/Conceptual_06.22.07.pdf
- Tritter, J. Q., & McCallum, A. (2006). The snakes and ladders of user involvement: Moving beyond Arnstein. *Health Policy (Amsterdam, Netherlands)*, 76(2), 156-168. doi:S0168-8510(05)00131-4
- Ulrich, A., Thompson, B., Livaudais, J. C., Espinoza, N., Cordova, A., & Coronado, G. D. (2013). Issues in biomedical research: What do Hispanics think? *American Journal of Health Behavior*, 37(1), 80-85. doi:10.5993/AJHB.37.1.9
- University of Washington Center for Genomics and Healthcare Equity. (2012). *Reflections on partnering in genetic research*. Retrieved from http://genetics.ncai.org/files/Reflections_on_Partnering_in_Genetics_Research_05112012.pdf
- Upton, F., & DeGette, D. (2015, January 13). Can we find cures for 7,000 disease? *CNN*. Retrieved from <http://www.cnn.com/2015/01/13/opinion/upton-degette-cure-diseases/>
- Wertheimer, A. (2014). The social value requirement reconsidered. *Bioethics*. doi:10.1111/bioe.12128
- Yarborough, M., Fryer-Edwards, K., Geller, G., & Sharp, R. R. (2009). Transforming the culture of biomedical research from compliance to trustworthiness: Insights from nonmedical sectors. *Academic Medicine: Journal of the Association of American Medical Colleges*, 84(4), 472-477. doi:10.1097/ACM.0b013e31819a8aa6

APPENDICES

APPENDIX 1: Johns Hopkins Bloomberg School of Public Health IRB Notices

A: Original IRB Approval Notice



FWA #00000287

JHSPH Institutional Review Board Office

615 N. Wolfe Street / Suite E1100
Baltimore, Maryland 21205
Office Phone: (410) 955-3193
Toll Free: 1-888-263-3242
Fax Number: (410) 502-0584
E-mail Address: irboffice@jhsp.h.edu
Website: www.jhsp.h.edu/irb

INITIAL APPLICATION EXEMPT DETERMINATION NOTICE

Date: August 7, 2013

To: Nancy Kass, Sc.D.
(Lauren Ellis)
Department of Health Policy & Management

Re: **Study Title:** "Exploring Patient Engagement in PCORI-Funded Health Research"
IRB No: 00005238

The JHSPH IRB reviewed the above-referenced new application on **August 5, 2013**. We have determined that the human subjects research activity described in your application meets the criteria for Exemption under 45 CFR 46.101(b), Category (2).

This determination is inclusive of the following documentation:

- Research Plan (V1, July 31, 2013)
- Oral Consent Form: Cognitive Interview Participants (V2, 8/5/13)
- Oral Consent Form: Interviews with Patients (V2, 8/5/13)
- Oral Consent Form: Interviews with Researchers (V2, 8/5/13)
- Disclosure Form: Observation of Community Outreach (V2, 8/5/13)
- In-Depth Interview Guide: Patients (V2, 8/5/13)
- In-Depth Interview Guide: Researchers V2, 8/5/13)
- Phone Script: Patients (V2, 8/5/13)
- Phone Script: Cognitive Interview Participants (V2, 8/5/13)
- Phone Script: COEC Community Relations Coordinators (V2, 8/5/13)
- Phone Script: Eligible Studies and Principal Investigators (V2, 8/5/13)
- Initial Email: Patients (V2, 8/5/13)
- Initial Email: Eligible Studies and Principal Investigators (V2, 8/5/13)
- Initial Email: COEC Community Relations Coordinators (V2, 8/5/13)
- Initial Email: Cognitive Interview Participants (V2, 8/5/13)
- Second Email: Patients (V2, 8/5/13)
- Second Email: Eligible Studies and Principal Investigators (V2, 8/5/13)
- Second Email: COEC Community Relations Coordinators (V2, 8/5/13)
- Second Email: Cognitive Interview Participants (V2, 8/5/13)

Any change to the research activity must be submitted to the IRB before implementation to ensure that it does not change the Exempt determination. The IRB does not require continuing review or submission of a progress report for studies determined as exempt from federal regulations. Every three (3) years from the date of exempt determination, you will be contacted for an update on whether or not to keep the exempt study active in our records.

If you have any questions regarding this action, please contact the JHSPH IRB Office at (410) 955-3193 or via email at irboffice@jhsphe.edu.

ES/teb

Attachment (1): Exempt Research Categories: Federal Regulations 45 CFR 46.101

**Exempt Research Categories
(Federal Regulations 45 CFR 46.101)**

1. Research conducted in established or commonly accepted educational settings, involving normal educational practices, such as (I) research on regular and special education instructional strategies, or (II) research on the effectiveness of or the comparison among instructional techniques, curricula, or classroom management methods.
2. Research involving the use of educational tests (cognitive, diagnostic, aptitude, achievement), survey procedures, interview procedures or observation of public behavior, unless: (I) information obtained is recorded in such a manner that human subjects can be identified, directly or through identifiers linked to the subjects; and (II) any disclosure of the human subjects' responses outside the research could reasonably place the subjects at risk of criminal or civil liability or be damaging to the subjects' financial standing, employability, or reputation.
3. Research involving the use of educational tests (cognitive, diagnostic, aptitude, achievement), survey procedures, interview procedures, or observation of public behavior that is not exempt under paragraph (b)(2) of this section, if: (I) the human subjects are elected or appointed public officials or candidates for public office; or (II) Federal statute(s) require(s) without exception that the confidentiality of the personally identifiable information will be maintained throughout the research and thereafter.
4. Research involving the collection or study of existing data, documents, records, pathological specimens, or diagnostic specimens, if these sources are publicly available or if the information is recorded by the investigator in such a manner that subjects cannot be identified, directly or through identifiers linked to the subjects.
5. Research and demonstration projects which are conducted by or subject to the approval of Department or Agency heads, and which are designed to study, evaluate, or otherwise examine: (I) public benefit or service programs; (II) procedures for obtaining benefits or services under those programs; (III) possible changes in or alternatives to those programs or procedures; or (IV) possible changes in methods or levels of payment for benefits or services under those programs.
6. Taste and food quality evaluation and consumer acceptance studies, (I) if wholesome foods without additives are consumed or (II) if a food is consumed that contains a food ingredient at or below the level and for a use found to be safe, or agricultural chemical or environmental contaminant at or below the level found to be safe, by the Food and Drug Administration or approved by the Environmental Protection Agency or the Food Safety and Inspection Service of the U.S. Department of Agriculture.

B: IRB Amendment Approval Notice



JOHNS HOPKINS
BLOOMBERG
SCHOOL of PUBLIC HEALTH

FWA #00000287

JHSPH Institutional Review Board Office

615 N. Wolfe Street / Suite E1100
Baltimore, Maryland 21205
Office Phone: (410) 955-3193
Toll Free: 1-888-262-3242
Fax Number: (410) 502-0584
E-mail Address: irboffice@jhsp.h.edu
Website: www.jhsp.h.edu/irb

AMENDMENT APPROVAL NOTICE

Date: October 31, 2013

To: Nancy Kass, ScD
(Lauren Ellis)
Department of Health Policy and Management

From: Luke C. Mullany, PhD, MHS
Chair, IRB-X

Re: **Study Title:** "Exploring Patient Engagement in PCORI-Funded Health Research"
IRB No: 00005238
Study Expiration Date: October 24, 2014

Based on review of the Amendment Application (received October 11, 2013) for the above-referenced study, the JHSPH IRB determined that the proposed changes described in the amendment no longer meets the criteria for Exemption under Category (2). Thus, the primary reviewer performed a review using an expedited process and approved the amendment request described below on October 25, 2013.

Approval of the research is for the period of October 25, 2013 to October 24, 2014. A Progress Report for continuing review must be submitted to the IRB Office no later than six weeks prior to the approval lapse date of October 24, 2014.

Single Reviewer <input checked="" type="checkbox"/> Convened <input type="checkbox"/> DHHS 46.110... <input checked="" type="checkbox"/> DHHS... <input type="checkbox"/> FDA 36.110... <input type="checkbox"/> FDA... <input type="checkbox"/> Category: 6 & 7 GWAS... <input type="checkbox"/>	Consent/Parental Permission Required From: Adult Participant... <input checked="" type="checkbox"/> LAR... <input type="checkbox"/> One Parent... <input type="checkbox"/> Two Parents... <input type="checkbox"/> Legal Guardian... <input type="checkbox"/> (Foster Care Children)	Form of Consent/Permission: Written Consent... <input type="checkbox"/> Waiver of Signature... <input checked="" type="checkbox"/> (Oral Script) Waiver of Informed Consent... <input type="checkbox"/> HIPAA Authorization... <input type="checkbox"/> HIPAA Waiver... <input type="checkbox"/> No Longer Enrolling... <input type="checkbox"/>	Study Site(s): U.S. <input checked="" type="checkbox"/> International <input type="checkbox"/> List Country(ies):
Vulnerable Populations: Children... <input type="checkbox"/> Foster Care Children... <input type="checkbox"/>	Assent Required From: No children (waived)... <input type="checkbox"/> Children aged: <input type="checkbox"/>	Pregnant Women/Fetuses 46.204... <input type="checkbox"/> Neonates 46.203... <input type="checkbox"/>	Sample Size: (screened plus enrolled) 62

DHHS 46.404 ... <input type="checkbox"/> 46.405 ... <input type="checkbox"/> 46.406 ... <input type="checkbox"/>	FDA 30.31 <input type="checkbox"/> 30.32 <input type="checkbox"/> 30.33 <input type="checkbox"/>	Form of Assent: Written <input type="checkbox"/> Oral <input type="checkbox"/> Assent Statement in Parent Permission <input type="checkbox"/>	Prisoners 46.303 <input type="checkbox"/> 46.306 <input type="checkbox"/> Epidemiological Research..... <input type="checkbox"/>	Final Enrollment: Secondary Data Analysis: (# specimens/participants)
---	---	---	---	---

This amendment approval is for the following revisions to the above referenced study:

1. To make changes to the consent process to obtain oral consent from participants to proceed with the phone interview.
2. To make revisions to two interview guides due to feedback obtained during the pilot interview work.
3. To include use of two additional data collection instruments.
4. To include use of a concise and general thank you note that will be emailed to interview participants the day after the interview takes place.
5. To revise the research plan to clarify that the population from which interview participants will be recruited includes three researchers from Johns Hopkins Medicine and may include members of the public who receive care at Johns Hopkins.
6. To revise the research plan for changing the term "patient engagement" to "consumer engagement" and changing references to "patients" to "members of the public".

and is inclusive of the following revised or newly submitted documentation:

1. Research Plan (10/11/13)
2. In-depth Interview Guide: Researchers (V3, 10/11/13)
3. In-depth Interview Guide: Consumers/Members of the Public (V3, 10/11/13)
4. Demographic Questionnaire for Researchers (V1, 10/11/13)
5. Demographic Questionnaire for Patients (V1, 10/11/13)
6. Thank you email to participants (V1, 10/1/13)

As principal investigator of the research, you are responsible for fulfilling the following requirements of approval:

- 1) The co-investigators listed on the application should be kept informed of the status of the research.
- 2) Submit an Amendment Request Form for any changes in research. These changes in research are required to be reviewed and approved prior to the activation of the changes, with the following exceptions:
 - a) changes made to eliminate an apparent immediate hazard to the research participant may be instituted immediately and the JHSPH IRB should be informed of such changes promptly; and
 - b) changes to IRB Approved questionnaires, interview or focus group guides, other data collection or recruitment materials – limited to rewording to clarify meaning, correcting grammatical or typographical errors, or removing items that will not be used in the research.
- 3) Unanticipated problems involving risk of harm to participants or others that are related to the study procedures must be reported to the JHSPH IRB within 10 days of the time that the PI learns of such problems. A Problem Event Report Form must be submitted to the IRB immediately.
- 4) Only consent forms with a valid JHSPH IRB approval stamp or logo, with the correct IRB Approved version number and approval date may be presented to participants. All consent forms signed by subjects enrolled in the study should be retained on file. The Office of Graduate Education and Research conducts periodic compliance monitoring of study records, and consent documentation is part of such monitoring.
- 5) Federal regulations require review of approved research not less than once a year, unless a shorter period is determined by the IRB. **Therefore, a Progress Report for continuing review must be submitted to the IRB Office no later than six weeks prior to the approval lapse date. This will allow sufficient time for review of the application to be completed prior to the approval lapse date.** Failure to submit a Progress Report prior to the approval lapse date will result in termination of the study, at which point new participants may not be enrolled and currently enrolled participants must discontinue participation in the study. All ongoing research activities must stop immediately, including data analysis.
- 6) If your research involves international travel, please don't forget to register with the International Travel Registry <https://apps4.jhsph.edu/ITR/Default.aspx> so that the School may locate you in the event of an emergency.

If you have any questions regarding this action, please contact the JHSPH IRB Office at (410) 955-3193 or via email at irboffice@jhsph.edu

JP/feb

JHSPH IRB Amendment Approval Notice
Version #13, 21Feb12

APPENDIX 2: Extended Methods for Empirical Study

This qualitative study had two aims: (1) to characterize the experiences and perceptions of researchers regarding patient engagement in PCORI-funded research projects and (2) to characterize the experiences and perceptions of patients regarding patient engagement in PCORI-funded research projects. To achieve the aims of this project, qualitative interviews were conducted with 19 principal investigators (from 19 different projects) and 33 patients (from 18 of the same 19 projects). This study was reviewed and approved by the Johns Hopkins Bloomberg School of Public Health Institutional Review Board (JHSPH IRB) (Appendix 1).

DEVELOPMENT OF MATERIALS

Materials used for this empirical study included recruitment materials (Appendix 3), consent forms (Appendix 4), interview guides (Appendix 5), and questionnaires (Appendix 6) for both researchers and patients. The study materials were developed by the student investigator, reviewed by the student's dissertation advisor, and approved by the JHSPH IRB.

In order to test the interview guides, one in-person cognitive interview was conducted with a researcher from Johns Hopkins Hospital who had experience engaging patients and one cognitive phone interview was conducted with a patient serving on a research advisory board for a clinical research study at Johns Hopkins Hospital. The student investigator developed cognitive probes to assess how questions were interpreted and understood following the approach outlined by Beatty and Willis (2007). Cognitive interviews were audio-recorded but were not transcribed, and the student investigator

took extensive notes during the cognitive interviews to inform changes to interview questions, which consisted of re-wording and re-framing questions to make them more understandable.

The interview guides and short questionnaires were also reviewed by the Johns Hopkins Community Research Advisory Council (C-RAC) Research Review Committee, a group of volunteer Baltimore residents supported by the Community Engagement Program of the Johns Hopkins Institute for Clinical and Translational Research (ICTR) to evaluate and provide input on research projects (ICTR, n.d.). The student investigator presented the research aims and methods, discussed the research project, shared study materials, and fielded questions from Committee members. The Committee then provided written feedback to the student investigator after the presentation. Several changes were made to the interview guides and short questionnaires in response to feedback from the Committee. The student investigator gave a follow-up presentation to the Committee regarding how its feedback had been incorporated into the project. The Committee's feedback and a detailed list of the changes made in response to the feedback are located in Appendix 8.

STUDY SITE AND SAMPLE

Background Information about PCORI

Authorized by the Patient Protection and Affordable Care Act (2010), the Patient-Centered Outcomes Research Institute (PCORI) is an independent, nonprofit organization that funds comparative clinical effectiveness research (CER) (PCORI, 2014b), which is research that compares the benefits and harms of alternative methods to treat clinical

conditions or improve the delivery of care in the real world setting (Federal Coordinating Council for Comparative Effectiveness Research, 2009). A defining characteristic of PCORI's research portfolio is that it aims to answer patient-centered questions in order to help patients and other healthcare stakeholders make informed, evidence-based decisions (PCORI, 2014a).

As part of PCORI's effort to fund patient-centered research, PCORI is committed to the inclusion of patients and other healthcare stakeholders throughout the research process starting with PCORI's review of funding applications, which involves patient and other stakeholder reviewers along with scientific reviewers. Funding applications submitted to PCORI are required to include a plan for how patients and other stakeholders will be engaged in the research, and patient and stakeholder engagement is one of the criteria against which funding applications submitted to PCORI are evaluated (PCORI, 2015).

PCORI is set to spend over \$3 billion on research in this decade (Washington & Lipstein, 2011). Given PCORI's commitment to funding research that engages patients, PCORI was selected as the site for this empirical study investigating patient engagement in research. Projects from the first two PCORI funding awards, the Pilot Projects Grant Program and the Cycle I Awards, were selected for this study because these projects were already underway at the time this research was initiated, making them appropriate for investigation.

Establishing a Relationship with PCORI

The student investigator first became interested in the role of patients in health research and policy in January 2013. At that point, the student investigator developed a research proposal under the direction of her advisor. In May 2013, the research proposal was reviewed and approved by an interdisciplinary committee in conformity with the Johns Hopkins Bloomberg School of Public Health doctoral degree program requirements.

After receiving the school's approval to conduct her dissertation research, the student investigator contacted PCORI staff and arranged an informal meeting to learn more about patient engagement in PCORI research and to discuss having PCORI investigators and patients serve as the target sample for her research. During the meeting, PCORI staff expressed interest in the research project. They also informed the student investigator that PCORI was seeking to hire a student intern who could contribute to internal PCORI activities related to patient engagement. In July 2013, the student investigator was formally offered and accepted a part-time internship position with PCORI, reporting to Lori Frank, who was the Program Director of Engagement Research at PCORI at the time. Both PCORI and the student investigator understood the importance of keeping the student investigator's work as an intern for PCORI separate from her role as an independent student investigator conducting research as part of her dissertation. In her role as an intern, the student investigator assisted with synthesizing findings from the literature on patient engagement in research and contributed to data analysis for several evaluation projects including a project evaluating PCORI's merit

review process. She also helped contribute to the design and implementation of a longitudinal survey to measure patient engagement in PCORI's entire research portfolio.

In December 2013, the student investigator began conducting interviews with PCORI-funded investigators and their patient partners as part of her dissertation research. During the recruitment and informed consent processes, potential interview participants were notified that the project was being conducted as part of the student's PhD dissertation research and also that the student was an intern at PCORI. Recruitment emails also stated that PCORI was supportive of the student investigator conducting an "independent evaluation of PCORI-funded patient engagement." In the consent forms, interview participants were informed that PCORI would not have access to any information about which studies or individuals were recruited or interviewed. Names of subjects were not recorded on study materials or transcripts and any potentially identifying information was redacted from the transcripts. Further, PCORI did not have access to any data collected for this research project, and the student investigator retained full intellectual ownership of the data she collected as part of her dissertation research. The student investigator completed data collection for her dissertation research in May 2014 and subsequently focused her attention on data analysis and on the development of her empirical and conceptual manuscripts.

In June 2014, after 11 months serving as an intern at PCORI, the student investigator was formally offered and accepted a position as a part-time Program Associate for the Research Integration and Evaluation program at PCORI. In this role, the student investigator continued to contribute to PCORI work related to patient engagement in research under the supervision of Lori Frank. As with the internship

position, the student investigator's work related to her dissertation research was kept separate from her work as a PCORI employee. In February 2015, the student investigator left her position at PCORI so she could focus full time on completing her dissertation.

The student investigator's time working at PCORI gave her insights that helped her to cultivate a deeper understanding of the field of patient engagement, and she is grateful to have had such an opportunity; however, she also acknowledges that working at PCORI may have affected her results. Interview participants may have viewed the student investigator as part of PCORI—the researchers' funding organization—and so they may have been more inclined to give positive responses. The student investigator, however, took several steps to mitigate the potential for social desirability bias by explaining that the interview transcripts were confidential, de-identified, and would not be shared with PCORI.

Sampling from PCORI Pilot Projects

The PCORI Pilot Projects Grants Program, PCORI's inaugural funding award, was developed to lay the groundwork to support PCORI's future portfolio of CER (PCORI, 2011a). Unlike subsequent PCORI funding awards, the Pilot Projects were not required to be comparative studies; rather, they address a broad range of questions about developing instruments and methods for patient-centered outcomes research, establishing research priorities, engaging patients in research, and translating research findings into clinical practice. The Pilot Projects were not required to engage patients as a stipulation of funding because the focus on these projects was on methods to inform CER rather than CER itself; however, patient and stakeholder engagement was one of the criteria against

which funding applications were reviewed and prioritized, and the majority of the projects included some patient engagement. A total of 856 funding applications were submitted to the PCORI Pilot Projects Grant Program (PCORI, 2011b) and in June of 2012, PCORI awarded \$30 million in total funding to a slate of 50 two-year projects representing 23 states and the District of Columbia (PCORI, 2012a).

Purposeful sampling was used to develop a sample of PCORI Pilot Projects for inclusion into this study. The goal of the purposeful sampling was to include typical engagement approaches while also capturing variation with respect to several other aspects relevant to engagement. The funding applications of all 50 Pilot Projects were reviewed to identify studies with patient engagement and to facilitate the selection process. Studies with funding applications describing plans to engage patients were considered eligible. Patient engagement was defined as a process or activity in which researchers involved patients, patient representatives, community members, and/or caregivers in the research process in a way other than as subjects of the research, which is consistent with definitions of patient engagement in the extant literature (Hanley et al., 2003).

The majority of funding applications from the Pilot Projects—42 out of 50 (84%)—described plans for patient engagement and thus were considered eligible. The projects that were excluded consisted of an analytic methods study with no patient engagement plan, a study that exclusively engaged non-patient stakeholders such as hospital administrators, two studies that described engaging patients as research subjects but not in ways other than as subjects of research and thus not meeting the definition of patient engagement, and four studies that assessed one or more method(s) of engagement

but that did not engage patients beyond the method(s) under investigation. Studies assessing methods of patient engagement in which patients were also engaged beyond the patient engagement methods under investigation were considered eligible.

Eligible Pilot Projects—that is, projects with patient engagement—were categorized by the different kinds of approaches to engagement described in the funding applications. The main approaches described in funding applications and by which projects were categorized included advisory groups, focus groups/interviews, surveys, patient co-investigators, patient research team members, pilot testing, and novel methods. Because the goal of sampling was to capture typical approaches to patient engagement, a spreadsheet listing eligible projects by their engagement approach(es) was created to facilitate the selection of studies from each engagement approach. Further, a secondary goal of sampling was to capture diversity with respect to other characteristics relevant to patient engagement including the population engaged (e.g., healthy patients, sick patients, patients from advocacy organizations, lay patients, minority patients, elderly patients), whether researchers has a previous relationship with the patients they were engaging, whether projects were also engaging non-patient stakeholders (e.g., clinicians, policymakers, purchasers, payers), and the study topic (e.g., behavioral health, chronic disease, rare disease). This information was also extracted from the funding applications and entered into the spreadsheet to ensure that the study sample comprised a diverse and balanced selection of projects and captured the breadth of engagement approaches.

The selection, recruitment, and enrollment process took place in an iterative fashion. Initially, 10 studies that included all the engagement approaches and that captured variety across the other relevant characteristics described above were selected

for inclusion in this study. Principal investigators were recruited by email. If there was no response after two emails, principal investigators were then contacted by phone. During recruitment, potential participants were contacted up to four times (two emails followed by two phone calls). If principal investigators only had one way of being contacted, they were contacted up to four times by the mechanism available (phone or email). Each time that a principal investigator declined participation, the extraction spreadsheet for the eligible projects was reviewed and a suitable alternative project using the same approach or that was otherwise similar with respect to the sampling criteria was identified so as to maintain balance in the study sample. For example, a principal investigator of a project using focus groups with patients to solicit patient feedback on the content and design of a novel intervention declined participation because the patient focus groups had not yet been conducted at the time the investigator was contacted, so the investigator of an alternative project also involving focus groups with patients to solicit patient preferences on the content of a novel intervention was then recruited and enrolled in this study. Ultimately, 19 principal investigators of PCORI Pilot Projects were contacted; nine of the 19 investigators were enrolled in the study, and 10 of the 19 investigators declined participation. Of the 10 declining investigators, one principal investigator did not respond after four attempts and the others cited the following reasons: no time (n=3), IRB concerns (n=1), not yet engaging patients (n=1), not willing to have patients interviewed (n=2), and no longer able to contact patients whom they had engaged (n=2). The nine investigators who agreed to be interviewed directed Pilot Projects that used various typical engagement approaches and that were diverse across the range of characteristics cited above.

For every study that was included in the sample, a phone or in-person interview was conducted with the principal investigator and with one to two patients whom the principal investigator was engaging in his/her research. After principal investigators had agreed to participate in the study, they were asked to help identify two information-rich patients whom they had engaged as part of their PCORI project and who were different from each other in some meaningful way. Patients whom the principal investigator saw as being involved and interested in the patient engagement activities were considered information-rich. When two patients were interviewed, the way in which they were meaningfully different depended on the total number of patients engaged in the project and the make-up of the group of engaged patients but could include differences with respect to the following characteristics: affiliation with advocacy organizations, health status and experience with condition/disease being studied, previous experience being engaged in research, pre-existing relationship with the principal investigator, professional experience in the medical field, age, or gender.

Principal investigators were asked to assist with patient recruitment by first contacting patients to gauge their willingness to be contacted by the student investigator about participating in an interview. One principal investigator opted to notify all patients whom he was engaging about the possibility of being interviewed and then had the student investigator follow up with patients who expressed interest; however, the rest of the principal investigators recommended two patients and contacted only those two patients about participating in this study. For two of the nine projects, only one patient was contacted about being interviewed and thus was the only patient interview conducted for that project. In one of these cases, only one patient was actively involved in the

project; and in the other, the principal investigator only felt comfortable recommending one of the two patients engaged in the project due to the burden of participation combined with the poor health status of the other patient engaged in the project. A total of 18 Pilot Project patients were recruited; 16 enrolled and two declined participation because of lack of interest.

Sampling from PCORI Cycle I Awards

The Cycle I Awards, PCORI's first set of CER projects, include projects on PCORI's first four areas of national priorities for research: Assessment of Prevention, Diagnosis, and Treatment Options; Improving Healthcare Systems; Communication and Dissemination Research; and Addressing Disparities. Funding applications submitted to the Cycle I Awards were required to have a plan for engaging patients and other stakeholders, and engagement was one of the review criteria against which applications were evaluated. Nearly 500 applications were submitted to the PCORI Cycle I Award Program, and in December of 2012, PCORI awarded \$40.7 million in total funding to a slate of 25 three-year CER projects representing 17 states (PCORI, 2012b).

The purposeful sampling procedures for the PCORI Cycle I Awards took place after the majority of Pilot Project interviews were completed and followed the same procedures used for sampling the PCORI Pilot Projects. The goal of the purposeful sampling was again to include typical engagement approaches while also capturing variation with respect to several other attributes of the projects. The funding applications of all 25 Cycle I Awards were reviewed to identify studies with patient engagement and to facilitate the selection process. Studies with funding applications describing plans to

engage patients were eligible for inclusion, with patient engagement defined as a process or activity in which researchers involved patients, patient representatives, community members, and/or caregivers in the research process in a way other than as subjects of the research.

Upon review of the funding applications, all 25 Cycle I Awards were found to have plans for engaging patients and thus no studies from the Cycle I Awards were deemed ineligible. The Cycle I Awards were categorized by the different kinds of approaches to engagement described in the funding applications: advisory groups, focus groups/interviews, surveys, patient co-investigators, patient research team members, pilot testing, and novel methods. Because the goal of sampling was to capture typical approaches to patient engagement, a spreadsheet listing eligible studies by their engagement approach(es) was created to facilitate the selection of studies from each engagement approach. Further, a secondary goal of sampling was to capture variation with respect to other characteristics relevant to patient engagement including the population engaged (e.g., healthy patients, sick patients, patients from advocacy organizations, lay patients, minority patients, elderly patients), whether researchers has a previous relationship with the patients they were engaging, whether projects were also engaging non-patient stakeholders (e.g., clinicians, policymakers, purchasers, payers) and the study topic (e.g., behavioral health, chronic disease, rare disease). This information was also extracted from the funding applications and entered into the spreadsheet to ensure that the study sample comprised a diverse and balanced selection of projects and captured the breadth of engagement approaches.

The selection, recruitment, and enrollment process took place in an iterative fashion. Initially, 10 studies that included all the engagement approaches and that captured variation across the other characteristics described above were selected for inclusion in this study. During recruitment, potential participants were contacted up to four times (two emails followed by two phone calls or up to four times by a single mechanism for participants that only had one way of being contacted). Every time that a principal investigator declined participation, the extraction spreadsheet for the eligible studies was reviewed and a suitable alternative study using the same approach or that was otherwise similar with respect to other characteristics was identified so as to maintain balance in the study sample. For example, an investigator of a study involving a patient advisory panel with chronically ill patients from a national patient organization did not respond to recruitment emails or phone calls, so an investigator of an alternative study engaging two patient partners with a chronic condition, also from a national patient organization, was recruited and enrolled in this study. Ultimately, 14 principal investigators of Cycle I Awards were recruited; 10 of the 14 investigators were enrolled in the study, and the remaining four investigators declined participation. Of the four declining investigators, one did not respond after four attempts and the others that declined cited lack of time (n=1) and not willing to have patients interviewed (n=2) as reasons for declining. The ten investigators who agreed to participate directed Cycle I Awards that used various typical approaches to engagement and that were diverse across the range of characteristics cited above.

For every study that was included in the sample, a phone or in-person interview was conducted with the principal investigator and an attempt was made to interview one

to two patients whom the principal investigator was engaging in his/her research. After principal investigators had agreed to participate in the study, they were asked to help identify one to two information-rich patients whom they had engaged as part of their PCORI project and who were different from each other in some meaningful way. Patients whom the principal investigator saw as being involved and interested in the patient engagement activities were considered information-rich. When two patients were interviewed, the way in which they were meaningfully different depended on the total number of patients engaged in the project and the make-up of the group but could include differences with respect to the following characteristics: affiliation with advocacy organizations, health status and experience with condition/disease being studied, previous experience being engaged in research, pre-existing relationships with the principal investigator, professional experience in the medical field, age, or gender.

Principal investigators were asked to assist with patient recruitment by first contacting patients to gauge their willingness to be contacted by the student investigator about participating in an interview. Two principal investigators opted to notify all patients whom they were engaging about the possibility of being interviewed and then had the student investigator follow up with patients who expressed interest; however, the rest of the principal investigators recommended two patients and contacted only those two patients about participating in this study. One principal investigator who participated in an interview was subsequently unable to recommend patients for interview, citing difficulty contacting patients, and was eventually lost to follow up. Nineteen Cycle I Award patients were recruited, and 17 of the 19 enrolled. Of the two that declined participation, one cited lack of time and there was no response from the other.

INTERVIEW STRUCTURE

Each interview was conducted by the student investigator. Interviews were conducted in person (n= 25) or by phone (n=27). Prior to the start of each interview, oral informed consent was obtained. All interviews were audio-recorded and transcribed. The overall interview structure was the same for all interviews, which were conducted following an interview guide (Appendix 5); however, there was some variation in questions that were asked depending on the flow of conversation, and interview participants were free to elaborate on topics within the interview guide that were of greatest interest to them. At the start of the interview, principal investigators were asked to describe the research project and the way that patients were being engaged in the project, and patients were asked to describe the research project and the way that they had been involved in the project to date. After this introductory, grand tour question, follow-up probes were asked to gather additional background information and to explore key domains related to patient engagement. Both the patient and researcher interview guides contained four domains pertaining to patient engagement and specific questions within each domain were tailored to respondent type (patient or researcher):

1. *Background information*: This domain included general information about the research project and engagement approach including the research topic and methods, how researcher-patient relationships were established, and the nature of the engagement activities.
2. *Purpose of patient engagement*: This domain explored why researchers decided to engage patients, why patients decided to participate, what patients

took to be the purpose of engagement, what the expectations and specific goals of engagement were, and the extent to which goals had been achieved.

3. *Experience of patient engagement*: This domain consisted of probing questions on how researchers and patients felt the patient engagement activities went, including which procedures worked and did not work, what the challenges to and facilitators for engagement were, how patients influenced the projects, and what the effects of engagement on the research were.
4. *Beliefs about patient engagement more generally*: This domain included questions exploring beliefs about the purpose and usefulness of patient engagement in research in general, including at what stage in research is it appropriate to engage patients, for what kinds of research it is appropriate to engage patients, and how patient engagement may effect the research enterprise.

Participants were also asked to complete a short questionnaire (Appendix 6). The questionnaire was emailed to interview participants in advance of the interview. Some interview participants completed and returned the questionnaire to the student investigator via email prior to the interview; however, most participants completed the questionnaire after the interview. For phone interviews, participants were read the response options and the student investigator documented the participants' responses. There were two versions of the questionnaire—a researcher questionnaire containing seven questions and a patient questionnaire containing 10 questions. Both questionnaires contained demographic questions asking the respondent's race/ethnicity, age, and gender

as well as a question about whether the participant had ever participated in research as a research subject. The researcher questionnaire asked whether the researcher had previously engaged patients in prior research (if so, how many times), how many years the researcher had been an academic faculty member, and on how many grants the researcher had served as a primary investigator. The patient questionnaire asked patients if they had previously been engaged in a research project prior to this research (if so, how many times), whether the patient had a chronic condition, what stakeholder community the patient identifies with, the patient's highest level of education attained, and the patient's employment status.

DATA MANAGEMENT AND ANALYSIS

After each interview, the student investigator reviewed hand-written notes, entered the data from the questionnaire into the computer, and typed new analytic memos or added to existing analytic memos to note important topics or impressions. Interview audio-recordings were either transcribed by the student investigator or sent to a transcription service for transcription. Identifying information was redacted from all interview transcripts. Each interview transcript was checked against the full audio recording in order to identify and correct errors. The content of each interview transcript was also reviewed in order to identify areas to improve for future interviews and to identify emergent themes.

The approach to data analysis most closely followed a qualitative descriptive approach, which aims to generate a comprehensive description of the event under investigation (Sandelowski, 2000). Qualitative analysis of the interview data was an

iterative process with interviews conducted until informational redundancy was reached. The interview questions were used as a starting point for developing a deductive analytic coding scheme, with additional inductive codes added based on salient topics identified during the review of transcripts. All codes were then organized into thematic families and assigned descriptions. The draft coding scheme was applied to a subset of transcripts, and the codes and code families were further refined before applying the finalized coding scheme to all transcripts. Code families were reviewed and coded segments of text were compared to identify patterns and main themes. The coding scheme can be found in Appendix 6.

To test the reliability of the coding scheme, a second coder was trained on the coding scheme and independently applied codes to a total of six transcripts. The double-coded transcripts were compared and percent agreement was calculated. There was 80% agreement between coders, with the majority of discrepancies being where the primary coder applied a code and the second did not apply any code. There were some instances where the coders applied different codes from the same code family to the same section of text, but there were no instances in which the coders applied codes from different code families to the same section of text.

Data from the questionnaires were entered into a computer software program and analyzed to understand the demographics of the study sample and assess relationships with the qualitative findings. The qualitative software program ATLAS.TI (1999) was used to manage and organize the qualitative data.

REFERENCES

- ATLAS.ti. (1999). (Version 7 ed.) [Computer Software]. Berlin: Scientific Software Development.
- Beatty, P. C., & Willis, G. B. (2007). Research synthesis: The practice of cognitive interviewing. *Public Opinion Quarterly*, 71(2), 287-311. doi:10.1093/poq/nfm006
- Federal Coordinating Council for Comparative Effectiveness Research. (2009). *Report to the President and the Congress*. Washington, D.C.: Department of Health and Human Services.
- Hanley, B., Bradburn, J., Barnes, M., Evans, C., Goodare, H., Kelson, M., . . . Wallcraft, J. (2003). *Involving the public in NHS, public health and social care research: Briefing notes for researchers* (2nd ed.). Eastleigh: INVOLVE Support Unit. Retrieved from [http://www.twocanassociates.co.uk/perch/resources/files/Briefing%20Note%20Final_dat\(1\).pdf](http://www.twocanassociates.co.uk/perch/resources/files/Briefing%20Note%20Final_dat(1).pdf)
- Patient-Centered Outcomes Research Institute (PCORI). (2011a, September 30). PCORI funding announcement: Pilot Projects Grants. Retrieved from http://www.pcori.org/assets/PCORI-Pilot-Projects-Funding-Announcement-Amendment-1-_v2_-09302011.pdf
- Patient-Centered Outcomes Research Institute (PCORI). (2011b, December 5). Patient-Centered Outcomes Research Institute receives 856 applications for Pilot Projects Grants Program. Retrieved from <http://www.pcori.org/content/patient-centered-outcomes-research-institute-receives-856-applications-pilot-projects-grants>
- Patient-Centered Outcomes Research Institute (PCORI). (2012a, June 18). PCORI announces Pilot Projects Program awards recipients, research topics. Retrieved from <http://www.pcori.org/content/pcori-announces-pilot-projects-program-awards-recipients-research-topics>
- Patient-Centered Outcomes Research Institute (PCORI). (2012b, December 18). PCORI announces funding for first comparative effectiveness research projects. Retrieved from <http://www.pcori.org/content/pcori-announces-funding-first-comparative-effectiveness-research-projects>
- Patient-Centered Outcomes Research Institute (PCORI). (2014a, September 10). Research we support. Retrieved from <http://www.pcori.org/content/research-we-support>
- Patient-Centered Outcomes Research Institute (PCORI). (2014b, October 6). About us. Retrieved from <http://www.pcori.org/about-us>

Patient-Centered Outcomes Research Institute (PCORI). (2015, January 12). PCORI merit review process. Retrieved from <http://www.pcori.org/content/merit-review-process>

Patient Protection and Affordable Care Act of 2010, Pub. Law No. 111-148, 124 stat 727, §6301.

Sandelowski, M. (2000). Focus on research methods: Whatever happened to qualitative description? *Research in Nursing and Health*, 23(4), 334-340. doi:10.1002/1098-240X(200008)23:4<334::AID-NUR9>3.0.CO;2-G

The Johns Hopkins Institute for Clinical and Translational Research (ICTR). (n. d.). Community engagement program. Retrieved from <http://ictr.johnshopkins.edu/consulting/consulting-services/research-participant-and-community-partnerships-core/community-engagement-program/>

Washington, A. E., & Lipstein, S. H. (2011). The Patient-Centered Outcomes Research Institute--promoting better information, decisions, and health. *The New England Journal of Medicine*, 365(15), e31. doi:10.1056/NEJMp1109407

APPENDIX 3: Initial Recruitment Emails

A: Initial Recruitment Email for Principal Investigators

JOHNS HOPKINS BLOOMBERG SCHOOL OF PUBLIC HEALTH INITIAL EMAIL TO RECRUIT PRINCIPAL INVESTIGATORS

Study Title: Exploring Patient Engagement in PCORI-Funded Health Research

Principal Investigator: Nancy Kass, ScD

IRB Number: 5238

PI version number/date: Version 2 (8/5/2013)

To: Research Participants [Principal Investigators]
From: Lee-Lee Ellis, MA, doctoral candidate
CC: Nancy Kass, ScD, professor and PhD advisor
Email title: Request to Participate in Research

Dear [Name of Principal Investigator],

I am a doctoral student at Johns Hopkins Bloomberg School of Public Health, and I am writing to seek your willingness to be part of a qualitative interview study designed to evaluate patient engagement in PCORI-funded research projects. I am also currently serving as an intern at PCORI who is supportive of my conducting an independent evaluation of PCORI-funded patient engagement.

I am writing to you, as you are a recipient of a PCORI funding award, in the hope you would be willing to participate in a 45-60 minute interview. The interview will ask you to describe your experiences doing patient engagement in your PCORI-funded project.

We also hope to interview patients who have participated in these same patient engagement activities. The study will involve one 45-60 minute interview with principal investigators of PCORI-funded studies as well as interviews with two patients from each participating study.

I would greatly appreciate your considering being part of an interview. Please let me know your willingness by responding to this email or by contacting me by phone at [number]. I will then further explain the study and of course answer any questions.

I appreciate your time.

Sincerely,

Lee-Lee Ellis

Study Title: Exploring Patient Engagement in PCORI-Funded Health Research

Principal Investigator: Dr. Nancy Kass

IRB Study No.: IRB00005238

If you have any questions about your rights as a research participant, you may call the Johns Hopkins School of Public Health Institutional Review Board (IRB) at 410-955-3193, or 1-888-262-3242.

B: Initial Recruitment Email for Patients

**JOHNS HOPKINS BLOOMBERG SCHOOL OF PUBLIC HEALTH
INITIAL EMAIL TO RECRUIT PATIENTS/CONSUMERS**

Study Title: Exploring Patient Engagement in PCORI-Funded Health Research

Principal Investigator: Nancy Kass, ScD

IRB Number: 5238

PI version number/date: Version 2 (8/5/2013)

To: Research Participants [Patients/Consumers]
From: Lee-Lee Ellis, MA, PhD candidate
CC: Nancy Kass, ScD, professor and PhD advisor
CC: [Name of Principal Investigator]
Email title: Request to Participate in Research

Dear [Name of Patient/Consumer],

I am a graduate student at Johns Hopkins Bloomberg School of Public Health. I have been approved to do a project to do interviews with researchers and patients who took part in “patient engagement” activities.

I am writing to you because I believe that you helped researchers, or were part of an “engagement” activity in the last year or so. We are always trying to learn more about what works and what doesn’t when researchers involve patients and get their input.

I am trying to interview many patients who took part in activities like this to see what they think worked well and what didn’t. [Name of Principal Investigator] indicated that you are willing to be contacted about being interviewed for my project.

I am hoping to interview you once. The interview will last about 45 to 60 minutes. I can interview you in person or by phone. Please let me know if it is OK to interview you, or if you have questions. You can respond to this email, or you can call me at [number]. I will then explain the study and of course answer any questions.

I appreciate your time.

Sincerely,

Lee-Lee Ellis

Study Title: Exploring Patient Engagement in PCORI-Funded Health Research
Principal Investigator: Dr. Nancy Kass (IRB Study No.: IRB00005238)

If you have any questions about your rights as a research participant, you may call the Johns Hopkins School of Public Health Institutional Review Board (IRB) at 410-955-3193, or 1-888-262-3242.

APPENDIX 4: Participant Consent Forms

A: Consent Form for Principal Investigators

JOHNS HOPKINS BLOOMBERG SCHOOL OF PUBLIC HEALTH ORAL CONSENT FORM: INTERVIEWS WITH RESEARCHERS

Study Title: Exploring Patient Engagement in PCORI-Funded Health Research

Principal Investigator: Nancy Kass, ScD

IRB Number: 5238

PI version number/date: Version 2 (8/5/2013)

WHAT YOU SHOULD KNOW ABOUT BEING IN THIS STUDY

- You are being asked to join a research study.
- This consent form explains the research study and your part in the study.
- Please read it carefully and take as much time as you need.
- You are a volunteer. You can choose not to take part, and if you join, you may quit at any time. There will be no penalty if you decide to quit the study.
- During the study, I will tell you if I learn any new information that might affect whether you wish to continue to be in the study.

PURPOSE

You are invited to take part in a research study. The purpose of this study is to describe the experiences and views of researchers who have conducted patient engagement as part of their research funded by the Patient-Centered Outcomes Research Institute (PCORI). We will also be interviewing patients who took part in these patient engagement activities.

WHY YOU ARE BEING ASKED TO PARTICIPATE

You are being asked to participate in this study because you are a principal investigator whose PCORI-funded study involves patient engagement.

PROCEDURES

I am asking you, as the principal investigator of a PCORI-funded study that involves patient engagement, if you are willing to be interviewed about your experiences and perceptions of the patient engagement you conducted. I hope to interview the principal investigators from up to 20 different PCORI-funded studies. From each PCORI-funded study, I also hope to interview two patients about their experiences and perceptions of patient engagement.

In the interview, you will be asked questions about the patient engagement you conducted as part of your PCORI-funded study. I will ask you why you conducted patient engagement, your experience conducting patient engagement, and your views on how the

patient engagement went and what it achieved. I will also ask you a little about your views of patient engagement in general. You can skip any questions that you do not feel comfortable answering. With your permission, I will record the interview with a digital recorder. I expect that the interview will take 45 to 60 minutes of your time.

Because this research study seeks to explore patient engagement, I also plan to interview patients who have participated in the patient engagement activities of each participating PCORI-funded study. I have asked your willingness, or the willingness of someone on your staff, in recruiting two patients who have participated in your patient engagement activities. I have asked you to choose two patients who seemed particularly interested or involved in the patient engagement activities you conducted and who are different from each other in some meaningful way. I then asked that you or a member of your research staff contact these patients to see if they are willing to be contacted by me for this study. I have contacted or soon will contact patients who have agreed to be contacted about being interviewed as part of this study. If a selected patient is not interested in participating, I may ask you or the staff member to repeat the selection process until two information-rich patients are enrolled in this study.

RISKS/DISCOMFORTS

The risks of participating in this study are minimal. There are no physical risks to being in this study. I will be asking questions about your experiences and perceptions about the patient engagement you conducted. Some of these questions could make you feel uncomfortable. You do not have to answer any questions you would prefer not to answer. There is also a risk that someone may find out that you are in this study. I will do everything I can to prevent that. Your contact information will be stored separately from any notes, recordings, or transcripts resulting from this interview. I will not include any identifying information—such as your name, the name of your study, or your geographic location—in any notes or transcripts from the interview. PCORI will not have access to any information about which studies/individuals have been recruited for this study nor about who agreed to participate. You will not be named in any reports that are written on the basis of this research. Your contact information as well as the digital recording and transcript of the interview will be stored on a password-protected computer. Only the members of the research team will have access to this information, and they will not be allowed to share it with anyone else.

BENEFITS

There is no direct benefit to you from participating in this study. However, I hope the findings will be of interest to you and to others who conduct patient engagement in health research.

VOLUNTARY PARTICIPATION

Your participation in this study is completely voluntary. You do not have to agree to be in this study, and you may change your mind at any time.

WHO DO I CALL IF I HAVE QUESTIONS OR PROBLEMS?

Call the principal investigator, **Nancy Kass**, at [number] if you have questions or complaints about being in this study. Call or contact the Johns Hopkins Bloomberg School of Public Health IRB Office if you have questions about your rights as a study participant. Contact the IRB if you feel you have not been treated fairly or if you have other concerns. The IRB contact information is:

Address: Johns Hopkins Bloomberg School of Public Health
615 N. Wolfe Street, Suite E1100
Baltimore, MD 21205
Telephone: 410-955-3193
Toll Free: 1-888-262-3242
Fax: 410-502-0584
E-mail: irboffice@jhsph.edu

PERMISSION TO PROCEED

Is it okay to proceed with the interview?

**JOHNS HOPKINS BLOOMBERG SCHOOL OF PUBLIC HEALTH
ORAL CONSENT FORM:
INTERVIEWS WITH PATIENTS/CONSUMERS**

Study Title: Exploring Patient Engagement in PCORI-Funded Health Research

Principal Investigator: Nancy Kass, ScD

IRB Number: 5238

PI version number/date: Version 2 (8/5/2013)

WHAT YOU SHOULD KNOW ABOUT BEING IN THIS STUDY

- You are being asked to join a research study.
- This consent form explains the research study and your part in the study.
- Please read it carefully and take as much time as you need.
- You are a volunteer. You can choose not to take part, and if you join, you may quit at any time. There will be no penalty if you decide to quit the study.
- During the study, I will tell you if I learn any new information that might affect whether you wish to continue to be in the study.

PURPOSE

You are invited to take part in a research study. The purpose of this study is to describe the experiences and views of patients who have been involved in patient engagement activities as part of a research study. We will also be interviewing researchers who have conducted these patient engagement activities.

WHY YOU ARE BEING ASKED TO PARTICIPATE

You are being asked to be part of this study because you are a patient who has taken part in patient engagement activities conducted as part of a research study. These activities may have been focus groups, advisory board meetings, telephone conferences, meetings with researchers, or other kinds of consultation or collaboration between researchers and patients. We are also interviewing for this project the main researcher who held the activities in which you took part. That researcher recommended that we talk to you; I believe that the researcher also told us that you agreed to have me contact you about being in this study.

PROCEDURES

I am asking you, as a patient who has taken part in patient engagement, if you are willing to be interviewed about your experiences and views of patient engagement. I hope to interview up to 40 patients.

In the interview, I will ask you questions about the patient engagement in which you took part. I will ask you why you took part in the patient engagement, your experience during patient engagement, and your views on how the patient engagement went and what it

accomplished. I will also ask you a little about your views of patient engagement in general. You can skip any questions that you do not feel comfortable answering.

With your permission, I will record the interview with a digital recorder. I expect that the interview will take 45 to 60 minutes of your time.

RISKS/DISCOMFORTS

The risks of being in this study are minimal. There are no physical risks to being in this study. I will be asking questions about your experiences and perceptions about the patient engagement in which you took part. Some of these questions could make you feel uncomfortable. You do not have to answer any questions that you do not want to answer. There is also a risk that someone may find out that you are in this study. I will do everything I can to prevent that. Your contact information will be stored separately from any notes, recordings, or transcripts from this interview. I will not include any identifying information—such as your name, the name of the study in which you were involved, or your location—in any notes or transcripts from the interview. You will not be named in any reports that are written on the basis of this research. Your contact information, the digital recording, and the transcript of the interview will be stored on a password-protected computer. Only the members of the research team will have access to this information, and they will not be allowed to share it with anyone else.

BENEFITS

There is no direct benefit to you from being in this study. However, I hope the findings will be of interest to those doing patient engagement in research.

VOLUNTARY PARTICIPATION

Your participation in this study is completely voluntary. You do not have to agree to be in this study. You may change your mind at any time.

WHO DO I CALL IF I HAVE QUESTIONS OR PROBLEMS?

Call the principal investigator, **Nancy Kass**, at [number] if you have questions or complaints about being in this study. Call or contact the Johns Hopkins Bloomberg School of Public Health IRB Office if you have questions about your rights as a study participant. Contact the IRB if you feel you have not been treated fairly or if you have other concerns. The IRB contact information is:

Address:	Johns Hopkins Bloomberg School of Public Health 615 N. Wolfe Street, Suite E1100 Baltimore, MD 21205
Telephone:	410-955-3193
Toll Free:	1-888-262-3242
Fax:	410-502-0584
E-mail:	irboffice@jhsph.edu

PERMISSION TO PROCEED

Is it okay to proceed with the interview?

APPENDIX 5: In-Depth Interview Guides

A: In-Depth Interview Guide for Principal Investigators

JOHNS HOPKINS BLOOMBERG SCHOOL OF PUBLIC HEALTH IN-DEPTH INTERVIEW GUIDE: PRINCIPAL INVESTIGATORS

Study Title: Exploring Patient Engagement in PCORI-Funded Health Research

Principal Investigator: Nancy Kass, ScD

IRB Number: 5238

PI version number/date: Version 4 (12/16/2013)

Thank you for being willing to talk with me today. This interview is part of a project examining patient engagement activities conducted by recipients of PCORI funding awards in order to learn more about patient engagement. The study is part of my doctoral dissertation conducted for my PhD in health policy and bioethics at Johns Hopkins Bloomberg School of Public Health.

BACKGROUND INFORMATION & EXPERIENCE ENGAGING PATIENTS

1. Please tell me about your study and then where patient engagement fits in.

If not covered, ask the following probes:

- What types of activities have been done or are planned?
- How many engagement activities were conducted or are planned in total?
- How many patients have participated?
- Does the patient engagement for this study build on existing relationships you have with any patients or patient groups? If so, tell me more about that.
- Are you engaging any non-patient stakeholders? If so, are they participating in the same or separate activities as the patient stakeholders?

2. How has the patient engagement component of your study been going?

If not covered, ask the following probes:

- What has worked well for your patient engagement?
- What has not worked well?
- What has facilitated your patient engagement?

- What has hindered it? What has been challenging?
3. In a moment, I am going to ask you about what you have gotten out of involving patients in the study, but first I'd like you to tell me how involving patients has affected the research project.

If not covered, ask the following probes:

- In what ways, if any, has the patient engagement had a positive effect?
 - What have been the positive effects/impacts of involving people?
 - It's also important to consider the other side: what have been the negative effects/impacts?
4. As a researcher, what have you gotten out of the patient engagement component of your project?
 5. Did you do anything (or do you plan to do anything) to evaluate the patient engagement component of your project? If so, please tell me more about that.

EXPECTATIONS AND GOALS

1. Why did you decide to engage patients as part of your project?
2. What was the purpose of engaging patients as part of your research? What were the specific goals for the patient engagement?
3. Up to this point in your research, do you feel that the purpose and goals of engagement are being achieved? Why or why not?
4. What, if anything, went differently than you planned? Was this OK with you or not?
5. Looking forward, if you had the opportunity to design a new engagement plan, knowing what you know now, what changes, if any, would you make to your plan? For what reasons would you make each of these changes?

GENERAL BELIEFS

1. More generally, what does the term "patient engagement" mean to you?
2. What do you believe is the purpose of patient engagement in health research? Why do you think patient engagement is sometimes done in research studies?
3. For what types of studies do you think it is appropriate to engage patients?

- Why those types and not others?
 - Are there certain topic areas or fields or study populations for which patient engagement seems more appropriate? Why or why not?
 - At what points in the research process is it most appropriate to engage patients?
 - At what points in the research process, if any, is it inappropriate to engage patients?
4. What do you think patient engagement in health research can accomplish? For researchers? For engaged patients? For study participants? For the larger community? For the research enterprise?
5. In your view, do you think patient engagement in health research is a worthwhile activity? If so, please tell me what makes it worthwhile. If not, please tell me why.

**JOHNS HOPKINS BLOOMBERG SCHOOL OF PUBLIC HEALTH
IN-DEPTH INTERVIEW GUIDE:
PATIENTS/CONSUMERS**

Study Title: Exploring Patient Engagement in PCORI-Funded Health Research

Principal Investigator: Nancy Kass, ScD

IRB Number: 5238

PI version number/date: Version 3 (10/11/2013)

Thank you for being willing to meet with me today. This interview is part of a research project. We want to find out more about involving people in health research. The study is part of my research for my PhD at Johns Hopkins Bloomberg School of Public Health.

BACKGROUND INFORMATION AND EXPERIENCE BEING INVOLVED

1. Please tell me about the ways people have been involved in [PI]'s study.

If not covered, ask the following probes:

- How have you been involved?
- Why did you decide to get involved?
- How did you end up getting involved?
- Did you know [PI] or anyone from the research team before being involved?

2. Thinking back to times when you were involved in activities for the study, can you tell me what those activities/meetings were like?

If not covered, ask the following probes:

- What do you like best about being involved?
- What has been the worst about being involved?
- What are some things that [PI] does that make it work well?
- What are some things that [PI] does that make it not work well?
- What has been challenging?

3. In a moment, I am going to ask you about what you have gotten out of being involved in the study, but first I'd like you to tell me how you think your involvement has affected the research project.

If not covered, ask the following probes:

- What have been the good effects/impacts of involving people?
- It's also important to consider the other side: what have been the bad effects/impacts?

4. What, if anything, have YOU gotten out of being involved?

EXPECTATIONS AND GOALS

1. What were you expecting about being involved in the study going into it? Have your expectations been met?
2. What do you think were the specific goals [PI] had for involving people? Up to this point in the project, do you feel that that the goals are being achieved? Why or why not?

GENERAL BELIEFS

1. Why do you think researchers involve people/patients in their research studies?
2. What do you think involving people/patients in health research can accomplish?
3. For what types of studies should researchers involve people?

If not covered, ask the following probes:

- Why those types of studies and not others?
 - Are there certain research topics for which involving people seems more important?
4. Research involves many stages: coming up with what the study is going to look at, writing a grant proposal, designing the study methods, doing the research, analyzing the results, telling people about the results, and putting the results into practice. At what stages during research is it best to involve people? Are there times when it does not make sense to involve people?
 5. In your view, do you think involving people in research is a good thing to do? Please tell me why or why not.

APPENDIX 6: Participant Questionnaires

A: Questionnaire for Principal Investigators

JOHNS HOPKINS BLOOMBERG SCHOOL OF PUBLIC HEALTH DEMOGRAPHIC QUESTIONNAIRE FOR PRINCIPAL INVESTIGATORS

Study Title: Exploring Patient Engagement in PCORI- Funded Health Research

Principal Investigator: Nancy Kass, ScD

IRB Number: 5238

PI version number/date: Version 2 (11/21/13)

1. Have you ever had a research project that included community or patient engagement before your PCORI study?

☐ Yes

☐ No (If No, please skip question 1a)

1a. If yes, how many previous projects have you had that included community or patient engagement (not including this study)?

☐ 1

☐ 2

☐ 3

☐ 4 or more

2. Have you ever participated yourself as a research subject?

☐ Yes

☐ No

3. How many years have you been an academic faculty member?

☐ Less than 1 year

☐ 1-5 years

☐ 6-10 years

☐ 11 to 20 years

☐ More than 20 years

4. On how many grants (including this PCORI project) have you served as a primary investigator?

☐ 1-3

☐ 4-6

☐ 7-9

☐ 10 or more

5. With which of the following racial or ethnic groups do you most identify? (check all that apply)

☐ African American

☐ American Indian/Native American

☐ Asian/Pacific Islander

☐ Caucasian

☐ Hispanic or Latino

☐ Other

6. What is your age?

☐ 21-30 years

☐ 31-40 years

☐ 41-50 years

☐ 51-60 years

☐ 61 years or older

7. What is your gender?

☐ Male

☐ Female

B: Questionnaire for Patients

JOHNS HOPKINS BLOOMBERG SCHOOL OF PUBLIC HEALTH
DEMOGRAPHIC QUESTIONNAIRE FOR PATIENTS

Study Title: Exploring Patient Engagement in PCORI- Funded Health Research

Principal Investigator: Nancy Kass, ScD

IRB Number: 5238

PI version number/date: Version 3 (12/04/13)

1. Have you ever been involved in a research project in a way other than as a participant of the research?

☐ Yes

☐ No

2. If you have previously been involved, how many times have you done so (not including this one)?

☐ 1

☐ 2

☐ 3

☐ 4 or more

3. Have you ever participated in a research study?

☐ Yes

☐ No

4. Do you have a chronic disease or condition?

☐ Yes

☐ No

5. Which of the following groups do you most identify with? (check all that apply)

☐ Patient

- ☐ Patient advocate/representative
- ☐ Caregiver
- ☐ Family member/parent of patient
- ☐ Community member
- ☐ Other (please specify: _____)

6. What is your gender?

- ☐ Male
- ☐ Female

**7. Which of the following racial or ethnic groups do you most identify with?
(check all that apply)**

- ☐ African American/Black
- ☐ American Indian/Native American
- ☐ Asian/Pacific Islander
- ☐ Caucasian
- ☐ Hispanic or Latino
- ☐ Other

8. What is the highest degree or level of school you have completed?

- ☐ Some high school, no diploma
- ☐ High school graduate
- ☐ Trade/technical/vocational training
- ☐ Some college credit, no degree
- ☐ College graduate (Associate or Bachelor's)
- ☐ Graduate degree (Master's or Doctorate)

9. Which of the following best describes your employment status?

- ☐ Employed full-time
- ☐ Employed part-time
- ☐ Not employed and seeking employment
- ☐ Not employed and not seeking employment

10. What is your age?

☐ 20 years or younger

☐ 21-30 years

☐ 31-40 years

☐ 41-50 years

☐ 51-60 years

☐ 61 years or older

APPENDIX 7: Coding Scheme for Qualitative Analysis

Table A.1: Code Names and Definitions

CODE NAME	DEFINITION
1.0 GOALS OF PE	The Goals of Patient Engagement code family includes codes relating to the goals and expectations of patient engagement, whether goals of patient engagement have been achieved, and whether patient engagement is being evaluated in any way.
1.1 GOALS OF PE	Apply when participants describe the specific goals or tasks of engaging patients in this research project. This is in response to a question about the specific goals of engagement and is often double coded with a code from the "Specific Rationale/Effect of PE" code family.
1.2 EXPECTATIONS	Apply when participants describe their expectations for engagement, what they expected to happen or get out of engagement. This is in response to a direct question.
1.3 GOALS ACHIEVED OR NOT	Apply when participants describe whether or not or the extent to which the goals have been achieved. This is in response to a direct question after the participant has described the goals.
1.4 ASSESSMENT OF PE	Apply when researchers describe plans to evaluate or measure engagement in their own projects. This could include plans to conduct interviews, administer surveys, or get other types of feedback from the patients who have been involved. Also apply when researchers indicate that they do not have plans to assess engagement. This usually comes up in a response to a direct question about plans to assess engagement.
2.0 PE EXPERIENCE	The Patient Engagement Experience code family includes codes that describe aspects of the patient engagement process including the approach to patient engagement, the role of patients on the research project, challenges to patient engagement, facilitators for patient engagement, and ways in which patient engagement has personally impacted participants.
2.1 PRIOR EXPERIENCE	RESEARCHERS ONLY. Apply to comments about previous work engaging patients that provide an understanding for the researchers' views or for engaging patients in their PCORI project.
2.2 ENGAGEMENT APPROACH	Apply to descriptions about the engagement approach including what the approach is and what it does. Approaches may include advisory boards, patient co-investigators, patient members of the research team, focus groups/interviews, surveys, group forums, pilot testing, or novel methods. Example comment: "We have a family advisory board that meets once a month". This code is not to be applied every time the engagement approach is mentioned; rather it is just a way to categorize descriptions of engagement approaches in each project. This is different from the research approach, which should not be coded.
2.3 PATIENT ROLE	Apply to descriptions about patients' role on the research team or in the engagement approach. For example, "I am the patient-co investigator" or "The patient partner leads the family advisory board meetings." This code is not to be applied every time the patient role is mentioned; rather it is just a way to categorize descriptions of patient roles in each project.
2.4 CHALLENGES/	This is a broad code to be applied to comments about challenging

CODE NAME	DEFINITION
DOWNSIDERS	aspects or barriers to engaging patients in the research project. This could include poor communication, time, scheduling, recruitment of patient partners, issues with compensation, IRB issues. Downsides have to do with reasons researchers may not want to engage patients and negative effects from patient engagement. For example, saying that "research with patient engagement is a lot more work" or "additional time demand". This is usually in response to questions about what did not work so well, what are the lessons learned, and what are the downsides.
2.5 FACILITATORS	This is a broad code to be applied to comments on what worked well for engaging patients, what has been helpful, and what lessons have been learned. For example, this can include comments on the helpfulness of communication, shared leadership, the learning network, patient training, the use of a glossary, payment, and allowing extra time to meet with patients. This code should also be applied to comments about strategies for overcoming barriers and may be double coded with challenges/downsides. Something may be a barrier and an asset—for example, allowing more time is helpful to patients but slows the research process.
2.6 PERSONAL IMPACT FROM PE	Apply when researchers describe how engaging patients (either in this study or previously) has impacted the way they approach research, medical care, or another aspect of their career or personal life. Apply when patients describe that through participating in patient engagement, they have gained new knowledge or will now approach health care differently.
3.0 PATIENT-PI RELATIONSHIP	The Patient-Principle Investigator Relationship code family includes codes on how relationships were established, why patients decided to become involved, and the extent to which researchers or patients were trained for engagement. It also includes codes about tokenism in patient engagement, shared decision-making between researchers and patients, patients feeling like equals, patients having a sense of ownership over the project, the use of technical language when researchers talk to patients, and patients feeling included in the process.
3.1 HOW ESTABLISHED	Apply when researchers or patients describe how their relationship was established. This could include a description of previous interactions between the researchers and patients engaged in the project, preexisting personal relationships, or recruitment activities used to connect patient research partners and researchers.
3.2 WHY PATIENTS DECIDED	PATIENTS ONLY. Apply when patients describe why they decided to get involved in the project. This is usually in response to a direct question about why they decided to get involved.
3.3 TRAINING FOR PE	Apply to comments about conditioning, training, orienting, or educating patients or researchers with regard to patient engagement. Also apply to comments about training on research or science as needed for engagement.
3.4 TOKENISM	Apply when there are positive or negative comments about patients being engaged in a superficial or disingenuous manner. Apply when there are references to "checking a box" or "tokenism". Apply to comments that explicitly describe the engagement as <i>not</i> tokenism as

CODE NAME	DEFINITION
	well. For example, comments like "we are more than a checked box".
3.5 SHARED DECISION-MAKING	Apply to comments about researchers and patients making decisions together or how decisions are not made without first consulting or involving patients. Do not apply to text describing a study topic about shared decision-making or the importance of shared decision-making in clinical care; this code should only be applied to decision-making in the context of patient engagement in research.
3.6 EQUALITY	Apply to positive or negative comments about equality such as patients feeling equal/unequal, the presence of power differentials, or patients being treated with equal respect. This also includes descriptions of techniques to address inequality or promote equality during engagement activities such as using first names only to avoid hierarchy or other techniques to equalize roles.
3.7 OWNERSHIP	Apply to comments about patients having a sense of ownership over the project or an aspect of the project, researchers having to share ownership, or the significance of ownership.
3.8 JARGON/ FOREIGN CONCEPTS	Apply to comments about researchers using language/concepts that are inappropriate to patients. This includes comments about changing the language, creating a glossary of jargon terms, speaking each others' language, patients being unable to understand the scientific concepts researchers are saying.
3.9 FEELING LISTENED TO/INCLUDED	Apply to comments about patients feeling included as part of the research group; apply to comments about patients feeling like they have a voice that is heard. This includes comments on how researchers make an effort to involve the patients to get their feedback, and patients feeling that their feedback is valued, heard and incorporated into the project.
4.0 PATIENT CHARACTERISTIC	The Patient Characteristic code family includes codes on patients as advocates or typical patients, patients as having experience/expertise, and patients as representative of a larger patient population.
4.1 ADVOCATE PATIENT	Apply to comments about patients being professional/professional-leaning advocates. Patients may describe themselves as advocates or as having a background in research and advocacy, or researchers may describe a patient as an advocate or having been involved in an advocacy/patient organization. This code may be double coded with the typical/lay patient code, which is the contrast to the trained advocate patient. This code is not meant to capture patients who view themselves as advocates for themselves or another in a casual way (for example, "I advocated for my dad when he was in the hospital"), this is really meant to capture a significant level of advocacy experience or association with advocacy/patient organizations.
4.2 TYPICAL/ LAY PATIENT	Apply this code to positive or negative comments about engaging the layperson, typical patient, regular person, normal Joe, and so on. This code may come up in a discussion about the value of or contrast between trained advocate patients and typical patients.
4.3 EXPERTISE/ LIVED EXPERIENCE	Apply to comments about patients bringing their experience as a patient or as a working professional to engagement activities. This includes comments about patients sharing their experience of disease or as a patient, which may be different than how clinicians understand the disease or patients' experiences. This also includes comments about

CODE NAME	DEFINITION
	patients' professional experience being an asset to engagement (for example, "She is a marketing expert and so she helped us think about dissemination"). Lastly, this includes comments that refer to patients being experts because of their experience.
4.4 REPRESENTATIVENESS	Apply to explicit, positive or negative comments about selecting patients who are representative of a certain group/condition. Also apply to comments about issues of representativeness in the engagement sample. If someone is referred to as a "patient representative", do not code unless broader message is about concept of representativeness.
5.0 RESEARCH STAGE	The Research Stage code family includes codes that describe various aspects of the research process in which patient engagement takes place.
5.1 RESEARCH TOPICS OF AGENDA	Apply to comments about patient engagement in developing the research topic or setting the research agenda.
5.2 RESEARCH QUESTIONS	Apply to comments about patient engagement in developing the research hypothesis or questions.
5.3 PROPOSAL	Apply to comments about patient engagement in developing the research proposal (PCORI application).
5.4 RESEARCH TEAM	Apply to comments about patient engagement in establishing the research team. This could include patients using their network to find other patients to also serve on the research team.
5.5 STUDY DESIGN/ METHODS	Apply to comments about patient engagement in designing the study or choosing the study methods.
5.6 STUDY MATERIALS	Apply to comments about patient engagement in developing study materials. This includes interview questions, surveys, recruitment materials, and the like.
5.7 STUDY INTERVENTION	Apply to comments about patient engagement in designing or choosing the study intervention or aspects of the intervention.
5.8 STUDY OUTCOMES	Apply to comments about patient engagement in choosing or prioritizing study outcomes.
5.9 RECRUITMENT	Apply to comments about patient engagement in any aspect of study recruitment.
5.10 STUDY LOGISTICS	Apply to comments about patient engagement in miscellaneous aspects of study planning. This includes the IRB, consent procedures, and other logistical aspects of the study.
5.11 DATA COLLECTION	Apply to comments about patient engagement in collecting study data.
5.12 DATA ANALYSIS/ INTERPRETATION	Apply to comments about patient engagement in analyzing or interpreting data from the study.
5.13 DISSEMINATION	Apply to comments about patient engagement in any aspect of dissemination.
5.14 ENTIRE RESEARCH PROCESS	Apply when there is an explicit comment about patients being engaged at every stage or throughout the entire research process.
6.0 NATURE OF INFLUENCE	The Nature of Influence code family should be applied with the Research Stage code family, to the extent possible, to characterize what patient

CODE NAME	DEFINITION
	engagement at that stage entailed and how it may have influenced the research.
6.1 GAVE OPINIONS/ADVICE	Apply to comments about patients giving advice, feedback, opinions, or their perspectives on an aspect of the study during engagement activities.
6.2 CHANGED	Apply when the researchers or patients describe an aspect of the project changing as a result of patient input or when there are comments about aspects of the project being refined, tweaked, or modified as a result of patient involvement.
6.3 TESTED	Apply to comments about patients pilot testing the study intervention, study instruments, or other study materials. This could include patient advisors being given study materials (an iPad, a survey, or the intervention itself) to test how it works and how it can be improved before/while it is used for the study.
6.4 VALIDATED	Apply to comments about patients reinforcing/confirming the researchers' thinking or an aspect of the project. This could include using patients to test/check if the researchers' assumptions align with patients' thinking.
6.5 CRITIQUED/ASKED QUESTIONS	Apply when patients criticize, challenge, or ask questions with regard to an aspect of the research. This also includes comments about researchers wanting patients to be critical of the research approach in order to improve it, comments about patients seeing their role as asking penetrating questions to researchers.
6.6 OTHER INFLUENCE	Apply to other kinds of influence not captured by other codes in this family.
7.0 SPECIFIC RATIONALES/EFFECT	The Specific Rationales/Effect code family includes codes on various reasons cited for engagement and/or effects of patient engagement, as they relate to the SPECIFIC PCORI PROJECT.
7.1 INCORPORATE PT PERSPECTIVE	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients to get patient perspective, input, and/or experience. This includes comments about how patient feedback was necessary for the project.
7.2 ENHANCE LEGITIMACY	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients as a way to enhance the legitimacy or credibility of the research.
7.3 KEEP PT-CENTERED	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients in order to ensure that the research is patient-centered, focused on the patient, or as a reminder of how patients are affected.
7.4 ENHANCE RELEVANCE	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients to increase the relevance of the research to the patient population. This includes comments about engaging patients to make research more meaningful or useful to patients or more reflective of what patients think is important, their needs, or reality.
7.5 ENHANCE FEASIBILITY	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients to enhance the appropriateness or acceptability of an aspect of the study or to ensure that the research process is feasible/practical.
7.6 ENHANCE GENERALIZABILITY	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients in order to enhance external validity/generalizability or to reach a wider or more diverse group of patients.

CODE NAME	DEFINITION
7.7 ENHANCE VALIDITY/ QUALITY	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients in order to improve the quality of the research, the validity of the research findings, or to advance research more generally. This may include nonspecific comments about patient engagement making the research project better.
7.8 IMPROVE CARE	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients as a way to improve patient care (for either the engaged patients or the patient community more broadly). This is usually an aspirational or distal effect.
7.9 IMPROVE DISSEMINATION/ COMMUNICATION	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients in order to improve communication between researchers/clinicians and patients or to improve the dissemination and implementation of results.
7.10 SHOW RESPECT TO PTS	Apply to PCORI PROJECT-SPECIFIC comments about engaging patients in order to demonstrate respect to patients. This may include comments about patients deserving to be involved.
7.11 PCORI REQUIREMENT	Apply to PCORI PROJECT-SPECIFIC comments about researchers engaging patients because PCORI (or funders more generally) are interested in it or require it.
7.12 OTHER	Apply to PCORI PROJECT-SPECIFIC comments about rationales for or effects of PE that are not captured by other codes in this family.
7.13 INFERENCE	VALENCE CODE. Apply this code when the comment <i>implies</i> a rationale for or effect of engagement but does not explicitly reference the particular rationale. For example, if the logical conclusion of the influence of engaged patients is that it will make the results more relevant, code as "7.4 Enhanced Relevance" and then add this valence code to indicate that this is an inference the coder is making.
8.0 GENERAL RATIONALES/ EFFECTS	The General Rationales/Effects code family includes codes on various reasons cited for engagement and/or effects of patient engagement, as they relate to research in GENERAL (not specific to the PCORI project).
8.1 INCORPORATE PT PERSPECTIVE	Apply to GENERAL (not PCORI project-specific) comments about engaging patients to get patient perspective, input, and/or experience.
8.2 ENHANCE LEGITIMACY	Apply to GENERAL (not PCORI project-specific) comments about engaging patients as a way to enhance the legitimacy and/or credibility of the research.
8.3 KEEP PT-CENTERED	Apply to GENERAL (not PCORI project-specific) comments about engaging patients in order to ensure that the research is patient-centered, focused on patients, or as a reminder of how patients are affected.
8.4 ENHANCE RELEVANCE	Apply to GENERAL (not PCORI project-specific) comments about engaging patients to increase the relevance of the research. This includes comments about engaging patients to make research more meaningful or useful to patients or more reflective of what patients think is important, their needs, or reality.
8.5 ENHANCE FEASIBILITY	Apply to GENERAL (not PCORI project-specific) comments about engaging patients to enhance the appropriateness or acceptability of an aspect of the study or to ensure that the research process is feasible/practical.
8.6 ENHANCE	Apply to GENERAL (not PCORI project-specific) comments about

CODE NAME	DEFINITION
GENERALIZ- ABILITY	engaging patients in order to enhance external validity/generalizability or to reach a wider or more diverse group of patients.
8.7 ENHANCE VALIDITY/ QUALITY	Apply to GENERAL (not PCORI project-specific) comments about engaging patients in order to improve the quality of the research, the validity of the research findings, or to advance research more generally. This may include nonspecific comments about patient engagement making research better.
8.8 IMPROVE CARE	Apply to GENERAL (not PCORI project-specific) comments about engaging patients as a way to improve patient care (for either the engaged patients or the patient community more broadly). This is usually an aspirational or distal effect.
8.9 IMPROVE DISSEMINATION/ COMMUNICATION	Apply to GENERAL (not PCORI project-specific) comments about engaging patients in order to improve communication between researchers/clinicians and patients or to improve the dissemination and implementation of results.
8.10 SHOW RESPECT TO PTS	Apply to GENERAL (not PCORI project-specific) comments about engaging patients in order to demonstrate respect to patients. This may include comments about patients deserving to be involved.
8.11 PCORI REQUIREMENT	Apply to GENERAL (not PCORI project-specific) comments about researchers engaging patients because PCORI (or funders more generally) are interested in it or require it.
8.12 OTHER	Apply to GENERAL (not PCORI project-specific) comments about rationales for or effects of PE that are not captured by other codes in this family.
8.13 INFERENCE	VALENCE CODE. Apply this code when the comment implies a rationale for or effect of engagement but does not explicitly reference the particular rationale. For example, if the logical conclusion of the influence of engaged patients is that it will make the results more relevant, code as "8.4 Enhanced Relevance" and then add this valence code to indicate that this is an inference the coder is making.
9.0 GENERAL BELIEFS	The General Beliefs code family includes codes about the participants beliefs about engagement in general, not necessarily specific to engagement in the PCORI project.
9.1 STAGES FOR PE	Apply to comments about stages of research (for example, proposal development, dissemination) when patients ought to be engaged. This arises often in response to a direct question but may appear elsewhere in transcript. Sections of text coded here may also be double coded with "PE Early in Process" or "Stages Not for PE" as they are often discussed in the same thought. Do not apply codes from the "Research Stage" family to general beliefs about stages of research for patient engagement.
9.2 STAGES NOT FOR PE	Apply to comments about stages of research when patients ought NOT to be engaged. This arises often in response to a direct question but may appear elsewhere in transcript. Do not apply codes from the "Research Stage" family to general beliefs about stages of research for patient engagement.
9.3 PE AT EVERY STAGE	Apply to comments about the philosophical view that patients should be engaged in all stages of the research process. Note that this does not mean that patients must have the same level of engagement across the study, just that they should be involved throughout. Do not apply codes

CODE NAME	DEFINITION
	from the "Research Stage" family to general beliefs about stages of research for patient engagement.
9.4 PE EARLY IN PROCESS	Apply to comments about the philosophical view that patients need to be engaged in the beginning of the research process.
9.5 KIND OF RESEARCH FOR PE	Apply to comments about the kind of research (disease topic area or methodology) in which it is appropriate to engage patients.
9.6 CAN'T DO RESEARCH WITHOUT PE	Apply to comments about the philosophical view that research (or a certain kind of research like outcomes research) cannot be done successfully without engaging patients.
9.7 EVIDENCE OF PE NEEDED	Apply to comments about the lack of evidence on the effects of patient engagement and the need to show that patient engagement is worthwhile.
9.8 CULTURE AGAINST PE	Apply to comments about the negative culture around patient engagement: a long history of not engaging patients or a view among researchers that patients cannot meaningfully contribute to research.
10.0 METAPHORS	The Metaphors code family includes metaphors that were commonly mentioned.
10.1 IVORY TOWER	Apply to the analogy of researchers doing work in an ivory tower, isolated from the rest of the world. Often this very analogy is used, but the comment does not have to invoke "ivory tower" just the idea (for example, researchers working behind locked doors, engagement getting researchers off the beaten path).
10.2 VOICE AT TABLE	Apply when there is a reference to patients having a voice at the table. Do not apply to comments that mention the patient voice but not in the context of being at the table. For example, a patient may say that they feel like their voice matters to the researchers, this should NOT be coded as "Voice at the Table" and should instead be coded as "3.9 Feeling listened to".
10.3 MARKET PLACE	Apply to comments that compare engaging patients in research to consumers/users being involved in product development (for example, it is like a chef getting feedback from customers).

APPENDIX 8: Community Research Advisory Council (C-RAC) Review

A: Feedback from C-RAC Research Review Committee

Community Research Advisory Council (C-RAC)

October 28, 2013 Research Review Meeting

Table A.2: C-RAC Feedback on Patient Questionnaire
Question 1: What is your gender should be grouped with questions about race, ethnicity, education, and age. The question should not be the first item on the survey.
Question 2: Which of the following stakeholder groups do you most identify? Delete the word “stakeholder” from the question; Delete the word “patient” as a response option
Question 3 and Question 4: Consider deleting the word “patient” from these items
Questions 3, 4, 5: Should be added to the in depth interview; they aren’t demographics
Question 6: Delete this question. It does not fit with the other questions, seems out of place. May raise concern among the Institutional Review Board or in an audit.
Question 7: Add a separate question; to determine if a respondent identifies as Hispanic/Latino since this is an ethnicity; delete the term Non-Hispanic
Question 7: Consider adding additional options for Asian/Pacific Islander
Question 9: Change the 1 st response to 18-20 years; since all participants will be at least 18.

Table A.3: C-RAC Feedback on Patient Interview Guide
Introduction -Add a definition of patient engagement Consider deleting or rephrasing question 1c, it appears out of place All questions appear to be redundant and require rephrasing
Page 2 and Page 1 appear to be different versions of the same questions; Do you want the C-RAC to recommend which version to use. If so, we recommend that you use page 2. The questions on page 2 are easier to understand and will help you get more information.
Consider reorganizing the survey so that: Section 1- Beliefs about patient engagement in health research general Section 2- The purpose of patient engagement Section 3- Experience participating in patient engagement

Table A.4: Executive Summary of Discussion During C-RAC Research Review
<p>Background Information and Study Overview</p> <p>Presented by Lee-Lee Ellis, MA, Principal Investigator PhD Candidate, Johns Hopkins Bloomberg School of Public Health</p>
<p>Patient engagement in health research is a process in which researchers collaborate with/or obtain advice from the general public regarding the planning and implementation of research. A few examples of patient engagement in health research include community-based participatory research, community advisory boards, and focus groups. The stakeholders that researchers collaborate with/or obtain advice from are referred to as “patients”.</p>
<p>The Patient-Centered Research Outcomes Institute (PCORI) is an organization that funds health research. Researchers seeking funding from PCORI are required to submit patient engagement plans; describing how they will collaborate with/or obtain advice from patients throughout the planning, implementation, and dissemination of their study.</p>
<p>Researchers and funders recognize the value of patient engagement in research, yet few studies have been conducted to demonstrate the effectiveness of this approach or how it is viewed by patients and researchers.</p>
<p>Purpose of Research: The purpose of the study is to determine how patients and researchers view patient engagement in health research; how patient engagement impacts health research; and which types of studies benefit the most from patient engagement.</p>
<p>Methods: My study will include up to 20 PCORI funded projects. Three representatives from each study (1 researcher; 2 patients) will be interviewed. I will obtain oral consent from participants before the interview is conducted. The interview will include demographic questions and questions to determine patients/researchers perceptions of patient engagement in health research. There is very little risk to people who participate and no direct benefit. We hope that the data can be used to improve patient engagement in health research.</p>
Reason for Research Review
<p>I am asking the C-RAC to provide feedback on my patient Demographic Questionnaire and Interview Guide. In 6-12 months, I’d also like to share my study findings with the C-RAC.</p>
Interactive Discussion: Questions about Research Study
<p>Member 1: Will the interviews occur before or after the patient engagement has started? Lee-Lee: The interviews will occur after the patient engagement has started.</p>
<p>Member 2: Are you interviewing the people individually or as a group? Lee-Lee: Each person will be interviewed individually.</p>
<p>Member 3: Are you doing all of these interviews yourself? Lee-Lee: Yes.</p>
<p>Member 5: Are the interviews done in person or phone? Lee-Lee: Some interviews will be done in person and some by phone.</p>
<p>Member 6: Why are you recruiting two patients per project? That’s a small number. Lee-Lee: I have approval to interview 60 people; so I will interview three people from each study (1 researcher and 2 patients). Some projects may only have one or two patients involved.</p>

<p>Member 1: How will you select the two patients, in a manner that is fair and equal?</p> <p>Lee-Lee: I will select people who are different from each other in some way. For example, if one patient is a part of a disease group; the other patient won't be.</p>
<p>Member 4: Have you identified the studies you are reaching out to?</p> <p>Lee-Lee: Yes, I have a list of all the studies I could possibly include.</p> <p>Member 4: Will the studies be at Hopkins or other institutions?</p> <p>Lee-Lee: A few studies may be from Hopkins, but mostly other institutions all across the U.S.</p>
<p>Member 5: How are you recruiting?</p> <p>Lee-Lee: I will email the head researcher. If they are interested in participating, I will explain that I want to interview them and two patients engaged in the research study. I will ask the researcher to identify two patients who may be interested in the study.</p> <p>Member 7: Are you recruiting from all PCORI funded studies?</p> <p>Lee-Lee: No, I will be emailing researchers of a subset of PCORI funded projects.</p>
<p>Member 7: What type of patients are you recruiting?</p> <p>Lee-Lee: I am really recruiting members of the general public who serve as advisors or part of research of team.</p> <p>Member 7: Oh, these are stakeholders, like our C-RAC board. Why not call them advisors?</p> <p>Lee-Lee: Yes. I call them "patients" because PCORI calls them patients and I want to be consistent.</p>
<p>Feedback on the Demographic Questionnaire (organized by question or theme)</p>
<p>Question 1: What is your gender?</p>
<p>Member 8: Don't think the survey should start off with gender. Since the survey is not focused on gender identity.</p> <p>Member 5: Gender should be included with education, race, and ethnicity.</p>
<p>Question 7: To which racial or ethnic group(s) do you most identify?</p>
<p>Member 9: You should have a separate question asking people if they are of Hispanic/Latino descent since this is an ethnicity.</p> <p>Member 10: This should be a separate question because people of any race can be of Hispanic/Latino descent. A lot of African-Americans are of Hispanic/Latino descent.</p>
<p>Question 6: Do you consider yourself someone who has a chronic disease?</p>
<p>Member 5: Why are you asking if a person has a chronic disease? How is this related to the survey? Do you want to find out what type of chronic disease a person has?</p> <p>Lee-Lee: I am not trying to find out what type of chronic disease someone has; just if they have a chronic disease. Patients with chronic disease may be more likely to think patient engagement is a good idea compared to patients who do not have a chronic condition. I want to be able to see if their views are different.</p> <p>Lee-Lee: I have to leave the meeting early. Please continue to discuss.</p> <p>Member 3: The question is too long and could be written clearer.</p> <p>Member 1: She could change the question to: Do you have a chronic disease?</p> <p>Member 5: Some people may feel uncomfortable answering this question.</p> <p>Member 8: Asking people if they have a chronic health condition is invasive. I wonder if it is necessary to get this information to answer the research question.</p> <p>Member 11: She may want to delete the question. It seems beyond the scope of the</p>

study.
Member 5: Let's recommend that she remove the question.
Questions 3 and 4: Use of the term community or patient engagement activities.
Member 8: I'm not sure how familiar people will be with the words "patient", "stakeholder", and "community engagement". She will need to define them and include examples. She may want to delete the word "patient" from these questions.
Question 2: Which of the following stakeholder groups do you most identify?
Member 8: She may want to delete the word "stakeholder" from the question.
Member 11: She may want to delete term "patient".
Member 3: I agree the word is confusing. Everyday people do not think of the word "patient" as "research advisors". This could be confusing.
Member 4: Let's suggest that the term is deleted.
Question 9: What is your age?
Member 5: Change the response 20 years or younger to 18-20 years.
Feedback on the Interview Guide (organized by page)
Page 1
Member 9: The introduction should include a description of what patient engagement is and how the term patient will be used throughout the study.
Member 8: The wording should be more conversational.
Member 9: For question 1, the probes don't seem to go with the question.
Member 4: Question 1a or Question 1b should be the first question.
Member 5: She should delete or rephrase Question 1c. It doesn't fit with the survey.
Member 4: Questions 2a-2f; 3-7 appear to be redundant and are hard to understand. She should rephrase and condense these questions.
Page 2
Member 3: I like questions on 2 nd page. Those questions should be on page 1.
Page 2- Start the survey with questions on beliefs related to patient engagement
Member 8: She may want to start the survey by asking questions about the general beliefs about patient engagement in research.
Next Steps
Schedule a follow up meeting with Ms. Ellis to discuss feedback.
Member 11: We may want to hold a follow up meeting.
Member 5: I will prepare the minutes and incorporate our feedback into the surveys. I will also ask Ms. Ellis if she'd like a follow up meeting.

Changes made based on C-RAC Research Review feedback

Demographic Questionnaire:

1. Reordered questions in the order C-RAC suggested (this meant starting with the question about the participant's previous experience with patient engagement, rather than starting with the question about gender). This was a helpful suggestion because it made the questionnaire flow better.
2. I re-worded both questions about previous engagement so that I did not have to say "patient engagement"—this, again, was a very helpful suggestion as the participants may not be familiar with that jargon.
3. I re-worded the question about which stakeholder group people belong to so that I would not have to use the word "stakeholder". This cut down on my use of unfamiliar jargon.
4. I removed the question about living with a chronic disease or condition. From our conversation, it became clear that this question was confusing. Here is the new question I came up with instead: "Do you have a chronic disease or condition"? There was some debate about the reason for including this question, and I'd like to tell you why I want to include it and see what you guys think.
5. I re-worded the question about racial/ethnic groups in order to place the power with the participant, as you suggested. Instead of asking them which ethnic group(s) best describes them, I am asking them which ethnic group(s) they identify with—this was the C-RAC's recommended language.
6. The C-RAC noticed that I had left out a response for people under 20 years old. I changed that to include ages below 20 as I may have participants between 18 and 20 years of age.

Interview Guide

1. Big picture: What I heard repeatedly in our discussion and what came across in the written feedback was that the interview guide seemed **redundant and confusing** in places. I **really appreciated** this feedback, and have taken several steps to address these issues.
2. Based on feedback from C-RAC and from a practice interview, I have deleted some questions that were either repetitive or unclear (see Table A.5).
3. Based on feedback from C-RAC, a practice interview participant, and my advisor, I have rephrased questions that were confusing (see Table A.6)
4. Based on feedback from C-RAC, I have tried to improve the flow of the interview guide by reorganizing the guide to have probes that I will only ask if the topic has not already been covered. The new guide has streamlined questions with follow-up probes to ask only when needed.

5. I have removed all references to “engagement” and “patient engagement” and replaced with “involving” (Table A.7).
6. I have removed references to “patient” and replaced with “people”. I am going to take cues from the participant about which words they use to describe the people involved (e.g., community members, people like them, patient representatives, caregivers, community partners, people) (Table A.7).
7. Since the C-RAC recommended I reverse the order of the questions in the interview guide and start with general questions first, I tested this out in a practice interview. I am not sure whether it is best to start with GENERAL questions about being involved or to start with the participant’s EXPERIENCE. I plan to decide after conducting a few more interviews, and I would like to get C-RAC’s input on this.

Table A.5: Interview Questions That Have Been Deleted	
Deleted Interview Questions	Reason for Deleting
What does the term “patient engagement” mean to you?	Patient interview participants are not likely to be familiar with this term, so it will not be used during the interview.
Do you think involving people in health research is a worthwhile activity?	Similar question in a two-part question, deleted one of two (repetitive)
Are there certain topic areas or fields in health or patient populations when it’s most important to involve people? Why or why not?	Intuitive answer that would not provide any new information about patient engagement; also the question is long and confusing
What do you think was the purpose of involving people as part of the study?	Nearly identical to another question (repetitive)
How do you feel your involvement has been going?	Confusing question that did not get at what I was trying to ask
What has worked well?	Nearly identical to another question (repetitive)
What has not worked well?	Nearly identical to another question (repetitive)
In your opinion, what was achieved by the patient engagement? Was this in line with what you thought would be achieved?	Similar to another question (repetitive)

Table A.6: Interview Questions That Have Been Rephrased	
Original Interview Questions	Rephrased Interview Question
I'd like to hear about the patient engagement activities for [name of study]. Can you tell me about those?	Please tell me about the ways people have been involved in [PI]'s study?
What have you liked about it? What haven't you liked about it?	What do you like best about being involved? What has been the worst about being involved?
Has anything helped make the patient engagement work well? If so, please tell me more about that. Has anything made it not work so well? If so, please tell me more about that.	What are some things that [PI] does that make it work well? What are some things that [PI] does that make it not work well? What has been challenging?
How do you think the patient engagement had a good effect on the research project? How do you think the patient engagement had a bad effect on the research project?	I'd like you to tell me how you think your involvement has affected the research project? [Probe on good/bad if not covered]

Table A.7: Jargon Terms That Have Been Removed from the Interview Guide	
Original term	Replacement
Patient	People (will take cues from participant and can use other terms including patient, patient representative, community member, caregiver, patient partner)
Patient engagement	Involving people in research
Patient populations	n/a
Research enterprise	n/a
Research process	Research (with listing out the different stages from coming up with what the study is going to look at to telling people about the results and putting them into practice)

APPENDIX 9: Lay Person Summary

Learning About Patient Engagement in Research

Background

Health researchers are involving patients in research in a new way. They are inviting patients to be advisors or partners, and not just to be “subjects” in their research. More and more, researchers are asking patients to give opinions on research. For example, researchers may ask patients about what to study. And patients can help researchers make decisions. Patients can also look at study forms like the consent form or a survey to see if they make sense. Patients may even help do research. Asking for patients to help make studies better in this way is called *patient engagement*.

People are excited about patient engagement. Some groups that give money to do research want researchers to involve patients in ways like this. They think it is a good thing to do. Even though people are excited about patient engagement, there are still lots of things to learn about it. We need to learn about the best ways to involve patients. We need to know what the effects of patient engagement are. Last, researchers need to know when and why it is important to engage patients in their research studies.

Research Methods

We did a research project to learn more about patient engagement. This research project had two parts.

- First, we did interviews both with researchers and with patients involved in the researchers' work. The goal was to hear about what researchers and patients thought about how patient engagement was going. We also wanted to learn about its effects.
- Second, in part two of this project, we wrote about why patient engagement is important. Next, we share what we learned.

Research Results

Part One

We did 19 interviews with researchers and 33 interviews with patients. After doing the interviews, we learned how patients were involved in research. Some patients were on advisory groups, and some were part of the research team. Some researchers met with patients a lot and some only met with them a few times. Patients gave ideas and reviewed research materials. Patients' advice sometimes led to changes to the research and other times it did not. It could make the research better, more useful, or easier to do.

Sometimes patient engagement was hard. It was hard for researchers to find patients and meet with them. It was also more work and time for researchers. Some patients felt that it was hard for them to share ideas when they did not know a lot about the research topic.

Some things made patient engagement go well. It was helpful when researchers had clear goals and told patients what those were. Teaching patients about the research topic helped patients be able to give better feedback. Researchers liked working with

patients who had been involved in research before. Patients also said that it was helpful when researchers were kind and polite to them.

After doing the interviews, we believe that there are things that can make patient engagement go better. New policies can make it easier for researchers to involve patients. Researchers need training on how to involve patients. Patients need to learn more about research. Researchers can share and learn from each other so that researchers who have done engagement before can help researchers who are new to it. New programs can help researchers find patients who want to be involved. These things can improve patient engagement.

Part Two

A lot of people think that it is important to engage patients, but it is hard to explain why. After doing this work, we believe that there are four reasons why patient engagement is important. First, patients can tell researchers about what is important to them. This may change what the research looks at or how the research is done. In that way, the research might end up being more useful to patients. Second, patient engagement can help researchers keep their promises, since patients can keep asking researchers about things they said, early on, that they would do. Making sure researchers keep their promises can help patients have trust in researchers in the future. Third, patient engagement is a way for researchers to learn how to show respect to patients. Researchers always need to respect patients. But sometimes they don't know how, and if they talk to patients, the patients can give them advice about ways to be more respectful. Fourth, even if involving patients does not change the research, involving patients itself

shows that researchers care and want to hear what patients have to say. Some patients have been ignored in the past. Patient engagement is a way to show that researchers are not ignoring patients, are thinking of them, and want to hear what patients care about. These reasons make patient engagement important. They can help researchers decide when to involve patients. Patient engagement is often a good thing to do even if it is not required.

Conclusion

This research taught us about how, when, and why researchers involve patients. It showed what is hard about it. We came up with things to do to make patient engagement better. This project showed what makes patient engagement a good thing to do. The results may help guide researchers and groups that fund research. We still have more to learn, but we can build on what we learned from this project.

CURRICULUM VITAE

Lauren “Lee-Lee” Ellis

EDUCATION

- Ph.D. Johns Hopkins Bloomberg School of Public Health Baltimore, MD**
Doctor of Philosophy in Health Policy & Bioethics, June 2015
Dissertation Title: *Characterizing Patient Engagement in Research Funded by the Patient-Centered Outcomes Research Institute and Exploring the Moral Importance of Patient Engagement in Research*
- M.A. New York University Graduate School of Arts and Sciences New York, NY**
Master of Arts in Bioethics, May 2010
- B.A. Colgate University Hamilton, NY**
Bachelor of Arts in Philosophy, Cum laude, May 2008
Honors in Philosophy, Honors in Liberal Arts Core Curriculum

RESEARCH EXPERIENCE

- Patient-Centered Outcomes Research Institute (PCORI) Washington, D.C.**
Program Associate June 2014 – February 2015
Fellow July 2013 – June 2014
- Conducted literature reviews on topics related to patient engagement in research
 - Designed and managed original survey research on stakeholder engagement in PCORI research
 - Analyzed qualitative data for several projects including data collected from PCORI applicants, reviewers, funded investigators, and patients and stakeholders engaged in PCORI projects
 - Contributed to manuscripts for peer-reviewed publication and prepared presentations to internal and external audiences to disseminate research findings
 - Worked with senior staff to coordinate and manage research activities with external consultants
- Johns Hopkins Berman Institute of Bioethics Baltimore, MD**
Research Assistant August 2011 – Present
- Developed research materials, conducted interviews, and performed preliminary data analysis for a project studying ways to improve how the concept of randomization is explained to potential research participants during the informed consent process
- Johns Hopkins Berman Institute of Bioethics Baltimore, MD**
Research Assistant October 2013 – April 2014
- Co-authored a conceptual manuscript exploring three practical reasons for researchers to engage communities in their research for a project supported by the Johns Hopkins Center for AIDS Research

Johns Hopkins Hospital**Baltimore, MD***Project Leader* January 2013 – September 2014*Research Assistant* January 2012 – May 2012

- Conducted qualitative interviews and analyzed data as part of an interdisciplinary research team studying the experiences of surgical intensive care unit patients, their families, and their providers
- Oversaw three graduate students in subsequent rounds of data collection and analysis and directed the preparation of a manuscript for peer-reviewed publication

Johns Hopkins Bloomberg School of Public Health**Baltimore, MD***Research Assistant* February 2011 – December 2011

- Conducted a moral analysis of issues that arise in regard to international food aid
- Developed a framework and rapid assessment tool used to enhance fairness in community selection for food aid in the Democratic Republic of the Congo

Food and Drug Administration (FDA)**Silver Spring, MD***Graduate Intern* June 2011 – August 2011

- Developed an ethics-based justification for FDA's position on the inclusion of children who are wards of the state in medical research
- Contributed to FDA Draft Guidance for ethics review boards, drug trial sponsors, and researchers

Colgate University**Hamilton, NY***Research Assistant* August 2007 – December 2007

- Performed a literature review and analyzed data from focus groups with people from rural communities in northeast Africa
- Produced an analytic report on community perceptions of how HIV/AIDS is spread in South Sudan

Dana Farber Cancer Institute and Children's Hospital Boston**Boston, MA***Research Assistant* May 2006 – August 2006

- Performed advanced laboratory techniques as part of biomedical research on rare genetic bone marrow failure disorders

TEACHING EXPERIENCE AT GRADUATE LEVEL

Guest Lecturer, Community-Based Participatory Research Seminar	Spring 2015
Teaching Assistant, Ethics of Public Health Practice in Developing Countries	Spring 2014
Teaching Assistant, Research Ethics and Integration	2012-2014
Project Leader, Qualitative Research I: Theory & Methods	Spring 2013
Project Leader, Qualitative Research II: Data Analysis	Spring 2013
Teaching Assistant, Ethical Issues in Public Health	Spring 2013
Teaching Assistant, Research Ethics Intensive	Summer 2012
Teaching Assistant, Health Behavior Change	Fall 2012

HONORS

- Center of Excellence in Regulatory Science & Innovation (CERSI) Scholar** July 2014
Granted by the Johns Hopkins University and Food and Drug Administration
- June Culley Scholarship in Health Policy and Management** April 2014
Granted by the Johns Hopkins Bloomberg School of Public Health
- Marcia Pines Prize in Bioethics and Public Health** June 2011
Granted by the Berman Institute of Bioethics
- M. Holmes Hartshorne Memorial Award** May 2008
Granted by Colgate University

PUBLICATIONS/PRESENTATIONS

Peer Reviewed Journal Articles

Ellis L, Gergen J, Wohlgemuth L, Nolan MT, Aslakson R. Empowering the "cheerers": A qualitative study on the role of surgical intensive care unit nurses in enhancing family resilience. *American Journal of Critical Care* (forthcoming).

Forsythe L, **Ellis L**, Edmundson L, Sabharwal R, Rein A, Konopka K, Frank L. Patient and Stakeholder Engagement in the PCORI Pilot Projects: Description and Lessons Learned. *Journal of General Internal Medicine* (forthcoming)

Frank L, Forsythe L, **Ellis L**, Schrandt S, Sheridan S, Gerson J, Konopka K, Daugherty S. Conceptual and practical foundations of patient engagement in research at the Patient-Centered Outcomes Research Institute. *Quality of Life Research*. 2015 Jan 6 (Epub ahead of print). DOI: 10.1007/s11136-014-0893-3.

Holzer JK, **Ellis L**, Merritt MW. Why we need community engagement in medical research. *Journal of Investigational Medicine*. 2014;62(6):851-855.

Submitted Articles

Ellis L, Kass N. Characterizing patient engagement in PCORI-funded research to enhance understanding of its ethical and practical value: A qualitative study of principal investigators and patients (submitted)

Ellis L, Kass N. Challenges to and successful strategies for patient engagement in research (submitted)

Presentations

Ellis, L. Exploring ethical rationales for patient engagement in research. *American Society for Bioethics and the Humanities*. September 6-9, 2014. San Diego, CA.

Ellis, L. Sabharwal R, Forsythe L, Edmundson L, Rein A, Frank L. Assessment of patient and stakeholder engagement in the PCORI Pilot Projects. *AcademyHealth 2014 Annual Research Meeting*. June 8-10, 2014. San Diego, CA.

Ellis, L. Aslakson R. Traveling with patients and their families on the “long road”: A qualitative analysis of Surgical Intensive Care Unit nurse perceptions. *Anesthesiology and Critical Care Medicine Research Conference*. December 10, 2012. Baltimore, MD

VOLUNTEER WORK

Community Research Advisory Council (C-RAC)

Baltimore, MD

Council Member August 2013 – Present

- Attend regular meetings to review and provide input on research projects as part of a council supported by the Johns Hopkins Institute for Clinical and Translational Research
- Serve on the planning committees for biannual workshops on community-engaged research
- Provide content expertise for ongoing evaluations of the research review process and outcomes

Southern Madison County (SOMAC) Volunteer Ambulance Corps

Hamilton, NY

Director of Membership December 2006 – May 2008

EMT September 2005 – May 2008

- Volunteered 40+ hours a week as an on-call Emergency Medical Technician (EMT) for a rural, low-income county in central New York by providing patient care and transport in medical emergencies
- Managed 40+ volunteers in the ambulance service, organized bimonthly clinical trainings, and scheduled crew shifts each week to ensure continuous ambulance service coverage

PROFESSIONAL DEVELOPMENT

Brocher Foundation Summer Academy in Population Health

Geneva, Switzerland

Summer Student June 2012

- Selected as one of 40 graduate student scholars from around the world for a weeklong academy on ethics and policy issues regarding the distribution of human resources for health

Proficient in Microsoft Office Suite, ATLAS.ti, NVivo

Proficient in Spanish (conversational)